Table of Contents

Introduction ................................................................................................................................. 4
  Greg Simon, President, FasterCures / The Center for Accelerating Medical Solutions

Executive Summary ...................................................................................................................... 6

Session 1: Why FasterCures? ........................................................................................................ 9
  Mike Milken, Chairman, FasterCures, and Greg Simon introduce FasterCures

Session 2: What Stands in the Way? ........................................................................................... 15
Identifying the barriers to improving the pace of medical discovery
  Interviews with David Baltimore, Ph.D., FasterCures board member, President of the California Institute of Technology and 1975 recipient of the Nobel Prize; William A. Haseltine, Ph.D., President, Haseltine and Associates and Founder, Human Genome Sciences; and Miles Gilburne, Managing Member, ZG Ventures, LLC.

Session 3: Looking 20 Years Ahead – And Getting There in 10 ................................................. 23
Emerging opportunities in medical research

Sessions 4 & 5: Case Study Approaches to Accelerating Medical Solutions ................................. 27
  Prostate Cancer: Leslie D. Michelson, Vice Chairman and Chief Executive Officer, Prostate Cancer Foundation; David Agus, M.D., Research Director, Louis Warschaw Prostate Cancer Center, Cedars-Sinai Medical Center
  Breast Cancer: Nancy Brinker, FasterCures board member and Founder of the Susan G. Komen Breast Cancer Foundation; Susan Braun, President and Chief Executive Officer, Susan G. Komen Breast Cancer Foundation
  Alzheimer’s Disease: Leon J. Thal, M.D., Professor and Chairman of Neurosciences and Principal Investigator of the Alzheimer’s Disease Cooperative Study, University of California, Los Angeles
  Epilepsy: Warren Lammert, Founder and Chief Investment Officer, Granite Point Capital and Co-Founder, The Epilepsy Project and epilepsy.com; Jacqueline French, M.D., Professor of Neurology, The Neurological Institute, the University of Pennsylvania School of Medicine and Co-Director, Penn Epilepsy Center; and Orrin Devinsky, M.D., Professor of Neurology, Neurosurgery and Psychiatry, New York University (NYU) Medical Center and Director, NYU Comprehensive Epilepsy Center
  What Color Is Your Diet? Using Nutrition to Accelerate Medical Solutions: Dr. David Heber, M.D., Ph.D., FACP, FACN, Professor of Medicine and Director, Center for Human Nutrition, University of California, Los Angeles

Session 6: The Fast Track to Faster Cures: .................................................................................. 39
Initiatives and opportunities to improve research resources, research organizations, and the research environment

Closing Session ............................................................................................................................. 45
The First Annual FasterCures Lake Tahoe Retreat

Greg Simon, President of FasterCures

The first annual FasterCures Lake Tahoe “Retreat” was really more of an “attack” – an attack on the barriers that slow new cures for deadly and debilitating diseases and an attack on the assumption that the current system cannot be changed. FasterCures organized the attack with the best army possible: a group of 60 leaders from diverse walks of life dedicated to accelerating medical solutions.

Many of the FasterCures board members and guest participants touch lives every day in their work as physicians, medical researchers, heads of disease foundations, health experts, and healthcare entrepreneurs. Others have been touched themselves by disease – directly and indirectly – by their own and their loved ones’ experiences as patients and participants in clinical trials and health research. All of the participants have demonstrated their support of and dedication to accelerating medical research to create a better, healthier future for all of society.

Over the course of three days, the Tahoe Group analyzed the barriers to faster cures and examined ways to defeat those barriers in order to accelerate innovation and bring tomorrow’s discoveries into being today. The group heard from leading scientists and advocates discussing research and treatment efforts for prostate cancer, breast cancer, Alzheimer’s, and epilepsy. Whether in highly technical research presentations or moving personal stories of families struggling with disease, the unifying theme was how FasterCures could meet the challenges facing the medical research community with new thinking and bold action. And while perspectives varied on the best way for FasterCures to approach the issues we face, there was a palpable consensus among the group that FasterCures’ mission is vital to promoting and catalyzing a heightened pace of medical progress.

Through our discussions, it became increasingly clear that the first step in accelerating the path to faster cures is to identify, empower, and strengthen the communities of interest who can remove the barriers impeding progress. This group, assembled in the splendor of Lake Tahoe, became one of those communities determined to make a difference in the fight for faster cures. And, in fact, through their ideas and energy at our “attack,” they have begun to make a difference already.
Let me introduce a representative sample of the group in their own words – and encourage you to join our group in the coming year.

“What I try to do is increase the collaboration between hospitals, physicians, and insurance companies, which is just a little less daunting than trying to cure cancer.” Richard Merkin

“When I finished college, I thought I was going to be an English Teacher. But I’m my husband’s right hand person in his consulting business, which is in the food and food ingredient industries. We have five daughters, four grandchildren, a big family. We travel a lot. And I’ve had MS for thirty-one years. And I too am looking for a cure.” Marilee Kovacs

“I’m Vice President for Research and Policy at the Kauffman Foundation, where a subset of our entrepreneurship agenda – accelerating the commercialization of all kinds of technologies including medical technologies – is very much tied to the FasterCures agenda.” Bob Litan

“I’m the Director of the Stanley Medical Research Institute, where we are trying to find a cure for the two most severe mental illnesses today, schizophrenia and bipolar disorder.” Mike Knable

“I tell everyone that my goal is to try to cure cancer by Tuesday. And so I’m pushing hard to do whatever I can.” John Klacking

“When I talk about passion and love I have to think about it personally and that’s my wife and kids and friends. Professionally, it’s working with organizations that are doing incredible things to save lives and help kids and families reach their highest, our highest potential.” Craig Stewart

“My career was business-focused until I, the same year as Mike Milken, was diagnosed with prostate cancer. After that I began changing my path in life to help fight against disease.” Shmuel Meitar
Executive Summary

Barriers to improving the pace of medical discovery
The Tahoe Group identified barriers that affect research resources, the research environment, and the infrastructure of research organizations. Key themes emerged in this discussion: the need to better apply technology to the research enterprise, the vast importance of better collaboration and coordination in the research environment, and better communication about and support of the research enterprise as a whole. The consumer is playing an increasingly important role in this arena, and their involvement needs to be better acknowledged and addressed.

Future areas of promise for accelerating medical research
When asked to consider the areas of focus most critical to accelerating medical research, participants identified: stem cell biology, cell biology, gene therapy, better data collection systems, intramural incentives for collaboration, new disease models, science education, and a call to action for the public support of research.

Immediate opportunities for accelerating medical solutions
Participants proposed immediate steps to accelerate activities in a range of arenas:

- **To build research resources:**
  - Increase the number of people participating in clinical trials; and
  - Develop more high quality medical record databases and biorepositories for research.

- **To improve the research environment:**
  - Minimize the negative impact of the Health Insurance Portability and Accountability Act (HIPPA) on the conduct of research;
  - Centralize institutional review board (IRB) policies and approval processes for multi-site trials;
  - Reform the Food and Drug Administration (FDA) approval process by examining the incentive/risk ratio; and
  - Provide better incentives at research institutions for translational and breakthrough research.

- **To support research organizations:**
  - Radically change the way the National Institutes of Health (NIH) funds and conducts research in order to merge various research disciplines;
  - Consider new funding and management models; and
  - Transform NIH into a more goal-oriented research center.
Case study approaches to accelerating medical solutions

Case studies discussed research advances, but also cited the ongoing opportunities and challenges facing these disease states:

- **Prostate Cancer needs**: increased clinical trial recruitment; preventive medicine vs. reactive; better biomarkers for disease; more drugs in development and faster development timelines.

- **Breast Cancer needs**: better consistency of care throughout the United States; increased understanding of normal breast biology, the causation of breast cancer, and prevention (need better biomarkers); comprehensive needs assessment for breast cancer research; centralized database for research; funding mechanisms that encourage collaboration.

- **Alzheimer’s Disease (AD) needs**: better animal models; increased research funding; improved clinical trials infrastructure; more innovative research.

- **Epilepsy needs**: therapies that work for the 20–40 percent of those who do not respond to current therapies; change in FDA review policy for new epilepsy therapies; creation of an epilepsy clinical trial consortium network run by academic centers; new insights into mechanisms responsible for intractable epilepsy.

- **Nutrition advice**: Your health condition is 80–90 percent reliant on how you live your life, and nutrition plays a key role. In a nutshell: our diets have too much fat, too much sugar, too much starch, and not enough protein.
Session 1

Why FasterCures?

THE ORIGINS OF FASTER CURES

I believed in the force before George Lucas did...

College is an amazing time of life - freedom, time for reflection.

How do we get the other 99% of people to contribute to health care??

Begin in the 1990's to focus on HUMAN CAPITAL - 7/10 of assets $1 trillion.

The 21st Century will be a COMPETITION for this capital. The US, the only country with a net input in last 30 yr.

• The value to the US of CURING CANCER is $1.75 trillion. Heart disease.

New...•• Heart disease.

• Shortening the TIME to cure diseases is an PURPOSE.

Once a BELIEF becomes a REALIZABLE DREAM, a lot of things can happen!.

ISSUES

Science & Technology

Law & En

Finance & Economics

Social Issues & Ethics

• Why DC?

After 9/11 we couldn't protect our children with our WEALTH, a renewed focus on Washington.

• How is FasterCures different? (not disease, not focused)

The first step (faster) were more structural - how things are going, we need to make it work - efficient.
Comments by Mike Milken, Chairman of FasterCures

To set the stage for the Retreat, Mike Milken discussed some key issues affecting society today and his perspective on the search for cures.

The value of “human capital”
Beginning in the 1960s, I concluded that balance sheets were inaccurate and it was really the human capital that was crucial to a company’s success. I began to work on this theory of human capital through allocating capital to organizations based on their human capital – not their financial balance sheets. Gary Becker’s economic work in the seventies coupled with mine in business resulted in an understanding that the majority of the assets of our country were in its human capital.

The United States is not fighting hard enough to maintain and support U.S. human capital or doing enough to attract talent from other countries. Ours is the only country in the world that has had a significant net importation of human capital since World War II. And we sometimes forget how many businesses, how many of our Nobel Prize winners, how many of our scientists, how many people who have made a difference have come here over the last fifty years and what they’ve accomplished and how our country has benefited from that. So the risk of losing this flow of capital has long term implications.

An important aspect of maximizing the output of existing human capital is in recognizing the incredible potential for productivity of this country’s aging population – people in their sixties, seventies, eighties, and even nineties. At this moment, a baby boomer is turning 50 every eight seconds, entering the age range where, unless new treatments and cures are developed, vast numbers of valuable and productive members of society will be compromised by disease and early death. If we are to preserve our human capital – a significant portion of which is aging – we need to accelerate medical solutions.

Losses and gains
One in four Americans will die from cancer. I lost ten family members to cancer. It’s been a focus of our various family foundations for more than two decades. Cancer patients, when you average it all out, die 15 years before they were expected to have passed away. So we’ve lost 15 potential productive years in the area of cancer. On a productivity basis alone [meaning productivity lost to mortality or untimely/early death], the value to the U.S. economy of curing cancer is forty-six and a half trillion dollars, based on estimates made by the Milken Institute. The figures for heart disease are even higher. And this has nothing to do with the value of a human being on a qualitative basis. This is just productivity.
While the impact of disease is still very real, we sometimes forget just how far we’ve come. At the beginning of the last century, one in five American babies did not live to celebrate a fifth birthday. And the diseases causing the biggest problems in the early 1900s were pneumonia, tuberculosis, and diarrhea. Improvements in the first half of the 20th century resulted in an enormous increase in life expectancy, due in part to the improved management of common diseases.

What next?
Once you have a belief that something significant can happen or a realizable dream like President Kennedy’s “we’re going to put a man on the moon,” a lot of things can happen. We believe there’s a lot of opportunity for an organization like FasterCures to realize the dream of achieving medical solutions more quickly. FasterCures must shorten the time in discovering paths to new treatment, the time in developing those treatments, and the time in bringing these treatments to patients. FasterCures will be looking at ways to work with government, academia, not-for-profits, and industry to determine how we can live longer, healthier lives. And part of FasterCures’ focus will be in recruiting the human capital needed to get the job done.

“The need for speedier progress in biomedical research is clear: each year, more than one million Americans suffer heart attacks, 600,000 have a stroke, and more than 550,000 die from cancer. Even a single year of acceleration in medical solutions could have a significant impact on saving lives. The choice is ours: we can sit back and wait for more cures and better treatments on the current 14 to 17 year timeline from discovery to the deployment of therapies or we can address the barriers to progress now, once and for all.” Mike Milken

For more information on Mike Milken, please visit www.mikemilken.com
FasterCures’ mission is to accelerate the discovery, development and deployment of new treatments and cures for serious and deadly disease.

While this mission is a key concern on the agenda for many disease advocacy and research organizations, it is the sole agenda item for FasterCures. In fact, if organizations were trying to do this as part of their mission, they wouldn’t be able to fulfill their other missions. If you’re in a disease group, your mission is that disease. It is not to change the system. And yet, as every disease group runs into problems in the system, they solve it for themselves. But they don’t solve it for anybody else because that’s not their mission, and they don’t have the time, the money, or the people to solve problems across the board. That is our job at FasterCures.

FasterCures’ mission touches a wide range of individuals and organizations – advocacy, funders, think tanks, government, trade associations, patient support groups, professional associations, and research centers.

We are getting incoming requests for help from all kinds of disease groups, all kinds of medical centers, other non-profit organizations, and government offices, because they know that our mission is action-oriented.

We’re talking about some very complicated, very entrenched, very difficult issues. And let me make one thing very clear: we are not trying to bring something down to build something else up.

The current system where science today rides on the shoulders of the giants of yesterday has served this country very well in medicine, in physics, in engineering, and in any number of other disciplines. We’re not talking about destroying the system that got us here. We’re talking about adding another layer that will allow things to happen more quickly through goal-oriented and efficiency-oriented processes.

What we’re trying to do is build a pathway to help expand the system we have today. One of the pathways we are focusing on is how can we get patients – the ultimate recipient of all of our efforts in the health arena – more involved in clinical trials and beyond? Before clinical trials, how can we get a patient to volunteer to have their tissue studied in a tissue bank, to have their blood studied by people doing protein analysis, to have their medical records correlated and collated with millions of other people so we can see a pattern?

“Let me tell you the question I get every time I talk about FasterCures. Question number one, isn’t somebody already doing this?... Short answer, no.”

Greg Simon, President of FasterCures
Imagine the difficulty you would have understanding a football game if all you could see was one player at a time. When you put them all together as a team and you see a play run, you see the pattern, and you understand each person’s role.

“Now the next question we get is how are you going to do this? Fair question.”

But getting back to pathways, that’s where we are asking for your input. We have an enormous A to Z list of issues that have been put forward for us to focus on. How do we possibly focus on all of those issues at once? The answer is you can’t. So the reason you’re here, the reason we’re here, is because we’ve been thinking very hard about what our priorities should be. How do we attack those priorities? And how do those priorities help us attack the next priorities down the road?

“Why does it take so long to find cures? Consider this: The potential speed of a high speed train is 200 miles per hour, but the average speed of today’s train is 55 miles per hour. It’s not the speed of the train that holds us back, it’s the speed of the track. FasterCures builds faster tracks for faster cures.”

Greg Simon
“Someone mentioned to me that all the barriers we’ve listed are human-created. We’re not dealing with natural disasters. No one is blaming the weather for the lack of drug discovery. If humans created the barrier, humans can take it down.”  

Greg Simon
Identifying the Barriers to Improving the Pace of Medical Discovery

What are the barriers to improving the pace of medical discovery? The Tahoe Group engaged in a lively brainstorm of what is slowing research today and the steps FasterCures could take to address these challenges. The discussion resulted in the following suggestions, which generally fell into the categories of research resources, the research environment, and research organizations:

Ideas for improving resources for medical research:

- Examine data needs in research, and create a data acquisition system that addresses privacy concerns
- Look at models where electronic medical records are being utilized
- Assess ways to attract more students into science and examine the human capital issues associated with medical research
- Identify resource gaps (e.g., funding, organization of data, knowledge management, information technology tools needed to conduct effective research, communication, infrastructure)
- Find biomarkers for diseases
- Capitalize on the role of the media in raising awareness of research and advances in medical research

“To effect the changes we’re talking about is going to require significant changes in consumer behavior. Information technology can really help with those changes.” Miles Gilburne

Suggestions for improving research environments:

- Examine ways to foster better collaboration across research entities
- Examine the FDA approval process to determine areas that can be improved
- Identify and examine other regulatory and legal issues that impede research progress (e.g., HIPAA)
- Identify ways to bridge the gap between basic and clinical research
- Emphasize prevention in the discussion of health and disease

“There are many barriers to communication in research: the wrong incentives, counterproductive reward structures, work that’s motivated purely by profit, no effective teamwork models.” Diane Thompson
Recommendations for enhancing the infrastructure of *research organizations*:

- Integrate information technologies into medical research
- Change the NIH infrastructure to allow for more translational research
- Examine ways that the pharmaceutical research infrastructure can be improved
- Improve clinical trials and look at ways to involve international trials in the process

“More emphasis is needed on prevention: at the national policy level, in public education about understanding prevention and compliance, and in reimbursement for those participating.” *Alice Huang*

**The Path from Basic Research to Cure**

*Greg Simon’s interview with David Baltimore, Ph.D., FasterCures board member, President of the California Institute of Technology, and 1975 recipient of the Nobel Prize*

Greg: “What is the nature of our research establishment: Is it suited to deal with the barriers we’ve been discussing? If not, what can we do about it? Can you explain to us what you mean when you say that we need alternative research organizations in certain areas to do certain kinds of work?”

“I am a basic research scientist and love basic research. I fundamentally believe that the advances that lead to cures for diseases and understanding of disease and prevention of disease come from basic research.” *David Baltimore*

**The relationship between basic research and cures**

David: “As I began thinking about how to apply new techniques – particularly gene therapy techniques – to ameliorate the effects of and to cure disease, I realized that the majority of research organizations, including the NIH, do not come from a ‘curing disease’ perspective, but instead focus on researching the disease itself.”

He continued: “There is a time in the analysis of any given disease when it’s just not worth trying to focus on the cure of the disease because we’re not yet poised to do that. The example I give is neurologic disease, where we still do not understand the fundamental concepts of how the brain works. So to deal with the underlying causes
of those diseases is extremely difficult. However, in cancer research since the 1970s we have gained a basic understanding of why cancer occurs, what kinds of genes are involved, what is wrong with those genes, and how to get therapies to deal with those underlying genetic defects. Therefore, cancer is a disease state where research should now be patient- and cure-oriented.”

**Breakthrough models for understanding disease**
In some cases, there have been research organizations that have made a commitment to pursue cures. Dr. Baltimore’s example: “Up until about four years ago, the NIH had no research on a vaccine for AIDS. It gave money to people who said they wanted to do it. But it had never taken responsibility for finding a vaccine. Everybody knew it was the most important thing: the world needed it, the United States needed it. And the NIH took a novel step and established the HIV Vaccine Research Center – which is, in fact, already the leading organization trying to find vaccines against HIV. So it can be done, and it can be done within the NIH framework. But these organizations have to think differently.”

**What do we need: reorganization or new organizations?**
Dr. Baltimore maintains that research entities need to figure out pathways to maneuver around the barriers in order to focus attention on curing disease. The NIH spends approximately $28 billion per year. In order to maximize the impact of this investment, the infrastructure needs reorganizing.

“We don’t need to produce brand new institutions from scratch. Our research institutes, our universities, and particularly our medical schools have tremendous flexibility to do what they want. There are barriers to new approaches, but you can’t avoid them. You need to understand the barriers and find your way through them. And that’s what the most successful research scientists do.” David Baltimore

**The Challenges Facing Research Entrepreneurship**
Greg Simon’s interview with William A. Haseltine, Ph.D., President, Haseltine and Associates and Founder, Human Genome Sciences

Greg: “Tell us about the barriers you saw when you were in research and why you felt becoming an entrepreneur would allow you to address those issues more effectively in a different environment.”
A perspective on the research enterprise

Bill: “First of all, I agree with David Baltimore in that there is a lack of disease focus – real disease focus – in our national research effort. In fact, the goal is knowledge mostly for knowledge’s sake … to understand disease but not to cure disease. That has deep ramifications throughout the research enterprise in our universities and our not-for-profit research institutes. They follow that lead.”

Dr. Haseltine outlined the three opportunities in academia: “Treating patients, doing fundamental research, and conducting clinical trials of medicines that are being created elsewhere.” He continued, “But, if you are working within the academic realm, you cannot – and I would argue that you should not – be able to take knowledge and move it forward in its direct application on a commercial scale. So if you are really interested in the translation of knowledge to human benefit, you want to understand and participate in the private sector, which is what I ultimately decided to do.”

The relationship between entrepreneurship and innovation

Bill: “I believe that the pharmaceutical enterprise is broken in a fundamental way. Individual entrepreneurship – seeking to create new knowledge – is fostered in the academic arena, but when you begin to institutionalize it in corporations, you begin to get into areas of difficulty. The current pharmaceutical research enterprise – with 20,000 scientists working on research and development within a single company – does not allow much room for individual entrepreneurship. On the flip side, the structure of small start-up companies lends itself to individual pursuits more easily. Yet these companies do not have the same access to capital or the knowledge to bring their products to market. Therefore, a prevailing conflict exists that thwarts innovation.”

During a break in sessions, UCLA Medical School Dean Gerald Levey, M.D., and William A. Haseltine, Ph.D., enjoy coffee and conversation amidst the outdoor splendor of the Sierra Nevada College campus.
What was the “take home” lesson from the race to sequence the human genome?
“That race shows you that it’s always better to have competitive efforts than monopolized efforts.” Dr. Haseltine explained that when the project was being operated solely in the government arena, it was a well organized project that would have gotten an answer in a certain amount of time with a certain funding investment. But, when challenged by a private sector enterprise, the government responded remarkably quickly and, together, both enterprises were successful. He summarized: “In science, and probably in every other field of human endeavor, competition is good.”

Information Technology and the Online Audience
Greg Simon’s interview with Miles Gilburne, Managing Member, ZG Ventures LLC, Member of the Board of Directors of Time Warner, and former America Online executive

Greg: “Share with us your viewpoint of the online arena. Are the barriers we’ve identified in the medical research arena the same as you’ve seen in the areas you work in? And how can the societal shift towards online communications impact healthcare, and ultimately, what we are trying to do at FasterCures?”

Technology: A view of the future
Miles: “The lesson from personal finance was if you get a lot of consumers who say ‘this is the way we want to trade stock’ and ‘this is the way we want to pay bills,’ the industry has to react. There were a few institutions established in personal finance that adopted, yet there were those that didn’t. They became diminished, and new companies emerged. The same thing happened with music over the last couple of years.” Mr. Gilburne used this example to illustrate how consumers drive demand. He emphasized the importance of making healthcare information digital in the coming decade, and how the utility of this service to customers will help drive the adoption of digital practices across the healthcare industry.

And what does this mean for healthcare?
Miles: “Healthcare is one of the key examples of products and services that over the next decade or two is going to become increasingly digital in all sorts of different areas, including: how you get access to healthcare, how you get information, how you interact with other providers of healthcare, what your digital medical records look like and how you use your digital medical records.”

“You would think that there would be a good mix and match between the big companies that are good at some things and little companies that are good at others. On the other hand, big companies think they’re good at everything. And little companies, unfortunately, think they’re better at many more things than they are.” William A. Haseltine
To make progress in healthcare, Mr. Gilburne posed additional questions:

- Can we remotely monitor a patient’s health and provide that information to their doctors in order to lower healthcare costs? (a question the Department of Veterans Affairs is now raising)
- Could the creation of an online environment facilitate the remote healthcare for chronically ill patients?
- Can we change the economics of healthcare and guarantee healthcare insurance coverage by demanding more consumer responsibility for their health through online maintenance?
- Can we build incentives into the system that would lower costs and support reimbursement?
Session 3

Looking 20 Years Ahead – and Getting There in 10
Emerging Opportunities in Medical Research

“Scientific progress is achieved by visionaries. Whether Jonas Salk and the polio vaccine, or Watson and Crick and the structure of DNA, researchers push the scientific envelope to make giant strides forward,” opened Greg Simon. “The polio vaccine story is the perfect example of where we need to be headed. The former head of the National Cancer Institute, Sam Broder, said that left to its own devices, NIH would have created a million state-of-the-art iron lungs to help polio patients. But instead, Salk – an outsider to the system – created the polio vaccine. So, rather than spending billions of dollars to treat polio, we spent a hundred million dollars to cure the disease.”

Greg described areas of medicine that hold great promise, including automated computer diagnosis and disease identification, fully synthetic human organs, complete real-time health monitoring, personalized medicine, and pharmacogenetics.

The Tahoe Group, invited to brainstorm specific ways that medical research could be accelerated, suggested the following areas of focus:

**Stem Cell Biology**

In the words of one attendee, “the greatest advances will come from the field of stem cell biology, as it has the greatest promise to change medicine as we know it.”

The political context of stem cell research, however, is having a significant impact on young scientists who are not willing to enter into such a politically charged research arena with uncertain funding prospects. Realizing the potential gains from stem cell research will take enormous resources and attention.

“It’s the sexiest thing going in biology today!”

President of Haseltine and Associates and Founder of Human Genome Sciences
William A. Haseltine, Ph.D.; University of Pennsylvania Professor Jacqueline French, M.D.; SF&L Railway Company CEO Morris Kulmer; Founder of Granite Point Capital and Co-Founder of The Epilepsy Project Warren Lammert; and California Institute of Technology President David Baltimore, Ph.D., participate in an afternoon discussion about acceleration.
“When we created the first White House website, you could literally drive to every website on the net. And now, we have billions and billions of websites. So if that can happen in ten years, what can happen in science and biology in twenty? And how can we make them happen in ten?”  

Greg Simon

**Cell Biology**

It is not just stem cell biology that offers such promise, but also cellular biology. The real breakthrough will come when we begin to use cells therapeutically. “Long before we get to stem cells, we need to figure out how to use existing adult cells for therapeutic purposes. Behind that we’ll have the whole stem cell movement.”

**Gene Therapy**

Gene therapy is a critical avenue in medical research, proving an important aid to, among other areas, stem cell therapy efforts. Through gene therapy, stem cells can be isolated from a patient, modified through gene therapy, and put back into the patient while avoiding the rejection problems typical of non-stem cell related transplants. While there are obvious concerns about gene therapy, given its somewhat checkered past, applying new treatment concepts generated through this basic research will be critical to future medical advances.

**Better Data Collection Systems**

In economics, diversified modern portfolio theory is based on studying hundreds of data points over long periods of time. Unfortunately, while today’s computers can handle a lot more in terms of data volume, a unified widely accessible data set for research does not exist. Huge amounts of data are being generated but they cannot be “pooled” and then mined for research because they are in different formats, are proprietary, or are not electronic, among other challenges. The full attention of mathematicians and computer scientists should be called on to solve this issue of capacity functionality.

“Get the Intels of the world to think ‘medicine.’”

*During a break in sessions, UCSF Professor Emeritus of Neurosurgery Charles Wilson, M.D., and Founder and CEO of American Shared Hospital Services Ernest Bates, M.D., shared thoughts on the Retreat discussions.*
**New Disease Models**

Given the limitations of our current disease models, there is a need for better, more accurate, pre-clinical models of disease (both animal and computational). Having a greater variety of appropriate disease models available would benefit the development of drugs, biologics, and devices across all categories of disease.

“Currently we can cure diseases in mice many times over, and yet these findings aren’t consistently translating into cures for humans.” Melanie Leitner

**Science Education**

As one attendee put it, “the biology education today is not preparing an individual for the biology of tomorrow” – which will be more heavily computational, more systems oriented. The delayed gratification inherent in science deters students from pursuing science and may lead to a brain-drain away from science careers.

**Public Support of Research & A Call to Action**

The public clearly needs to play a role in accelerating medical research, by supporting funding but also by challenging the system and asking for more results. Public support for prevention and improved health care quality are key to system-wide change.
Sessions 4 & 5
Case Study
Approaches to Accelerating Medical Solutions
Case Studies: Prostate Cancer, Breast Cancer, Alzheimer’s Disease, Epilepsy, Nutrition

Nationally-known experts on four serious diseases – prostate cancer, breast cancer, Alzheimer’s, and epilepsy – as well as a nutritional expert, were challenged to think about the most promising research happening in their fields and the existing barriers to further and faster progress.

Prostate Cancer

Introduction by Leslie D. Michelson, Vice Chairman and Chief Executive Officer, Prostate Cancer Foundation (PCF)

Over the past 50 years, there has been enormous progress in fighting cardiovascular disease, cerebral vascular diseases, pneumonia, and other infectious diseases, but not as many advances in fighting cancer as a whole. As of 2001, cancer represented one-third (553,768) of all deaths in the United States. Breast and prostate cancer are the top two cancer diseases. Prostate cancer is the number one cancer in men, with one in six men acquiring it in their lifetime. A non-smoking man is more likely to get prostate cancer than lung, bronchus, colon, rectal, bladder, lymphoma, melanoma, oral, and kidney cancers combined.

The PCF strategy to find better treatments and a cure includes:

- Competitive awards which only require scientists to put together brief project descriptions with funds awarded in 90 days;
- A therapy consortium with eight leading prostate cancer research centers from around the country collaborating on research;
- Industry collaboration; and
- A scientific retreat where 400 leaders from academia, industry, regulatory bodies, and patient advocacy groups come together to share research hypotheses and results, and collaborate on next steps.

Fortunately, the prostate cancer death rate is declining, largely due to the fact that investment in prostate cancer research has risen. Without improvements in diagnosis and treatment, by 2015 the number of new prostate cancer cases will have increased from approximately 230,000 today to more than 300,000. The challenge now is not just in providing increasing research funding, but in determining how to spend that money in order to gain near-term results.

Leslie D. Michelson presents the barriers and breakthroughs in prostate cancer research.
Clinical trial participation in cancer research

One of the measures of progress in this arena is the degree to which patients are participating in clinical trials. Although breast and prostate cancer have similar prevalence, more than four times as many women participate in breast cancer trials as men participate in prostate cancer trials. Recent data from the NCI Cooperative Group Trials Enrollment shows that less than one percent of prostate cancer patients are participating in clinical trials.

Commentary by David Agus, M.D., Research Director, Louis Warschaw Prostate Cancer Center, Cedars-Sinai Medical Center

Dr. David Agus reviewed the top priorities in prostate cancer research. He began by discussing the need to use advances in genomics and proteomics to individualize therapy. He also cited the need to move medicine from a reactive mode to a preventive mode.

Dr. Agus emphasized the need to be able to move research findings quickly from the laboratory to patient application. The internet could play an important role in patients’ ability to access information immediately as it is discovered in a laboratory.

Dr. Agus outlined four priorities in the quest to cure prostate cancer:

- **The heterogeneity of prostate cancer needs to be decoded.** Fifteen percent of men are diagnosed with the disease, of which 15 percent die. We need to figure out who is at risk for the lethal phenotypes, develop new targets, and identify better markers.

- **Better clinical trial designs are needed to reduce the length, cost, and size of trials.** The Therapy Consortium must be transformed into a Clinical Development Consortium that can launch trials more rapidly and less expensively.

- **The number of drugs under development for prostate cancer must be dramatically increased.**

- **The speed with which new drugs can be tested also must be dramatically increased.**

Key research accomplishments of the PCF include:

- Aggregating a critical mass of talented and dedicated scientists;
- Creating a rapid-response, risk-taking, high-impact funding system;
- Influencing government to increase prostate cancer research funding from $25 million in 1993 to more than $500 million in 2003;
- Providing over $120 million to more than 1,100 projects in 100 laboratories around the world;
- Creating a Therapy Consortium;
- Creating the world’s largest collection of family clustered prostate cancer cases; and
- Creating a tissue bank and animal model network.
Breast Cancer

Introduction by Nancy Brinker, FasterCures board member;
Founder of the Susan G. Komen Breast Cancer Foundation

Nancy Brinker spoke about the genesis of the Komen Foundation and her role in a movement that has revolutionized the arena of breast cancer research. Her involvement began when her older sister, Susan, was diagnosed with breast cancer. It was the seventies, and they both faced a community where the words “breast cancer” simply were not spoken aloud and where little information was available. Ms. Brinker encountered a magnitude of fear – fear that kept women from asking questions and aggressively seeking treatment. As her sister went through treatment, she and Ms. Brinker often talked about how they would “do something” to help others facing a breast cancer diagnosis. Susan was convinced of her sister’s ability to speed up cancer research and to dispel the paralyzing fear surrounding the disease.

In 1980, Susan died of breast cancer at the age of 36. This loss, later compounded by Ms. Brinker’s own personal experience with breast cancer, gave her a unique – though sadly, not uncommon – perspective that she chose to address with action. And, she had made a promise to her sister.

The journey began with nine volunteers in her living room in 1981, a list of people to call for help, and $200. At that time, despite the fact that women were being diagnosed every day, no one wanted to talk openly about breast cancer, let alone give money. For ten years the organization struggled. Ms. Brinker knew that they needed to raise money efficiently and dispense it in an effective way, and she was determined to create an organization that operated with integrity.

And Ms. Brinker and her team persevered. The organization currently has 114 city-affiliates, each incorporated with nonprofit status.
Breast cancer is the third most frequent cancer in the world and the most common female cancer worldwide, accounting for 21 percent of all new cancer cases. In the United States, one in eight women will have breast cancer in her lifetime.

Progress has been made. In the last twenty years, the mortality rate has decreased with earlier detection through advances in screening, education and awareness. There are better treatments available and modest declines in mortality have been associated with some of the hormonal, biologic, and chemotherapeutic interventions in breast cancer.

However, major unanswered questions include how much is understood about normal breast biology, the causation of breast cancer, and prevention. What can we tell women who have a family history of breast cancer in terms of prevention? In the area of causation, how are inherited factors, biology, and the environment interrelated?

Biomarkers can improve the ability to predict cancer risk. A biomarker is a test performed on cells or blood to help determine whether chemotherapy, a vaccine, or preventive agent is working. What role can biomarkers play in prevention?

Komen Foundation funds are disseminated through the affiliates, and a portion of the funds they generate are allocated through research grants. Affiliates awarded $32 million in 2003 in areas such as basic clinical translation grants, post-doctoral fellowships, and interdisciplinary fellowships.

Ms. Braun suggested the following approaches to accelerating breast cancer research:

- **A comprehensive needs assessment for breast cancer research:** which researchers are doing what, what the needs are, and which unmet needs must be targeted.

- **A centralized database for past and present research.**

- **Novel funding arrangements.** The current funding structure contains few mechanisms for collaborative hypothesis generation but instead rewards “one” researcher for a discovery, thus driving researchers to working in silos.

- **Collaboration to galvanize different specialties and disciplines so that a fresh look can be given to breast cancer problems.**
Alzheimer’s Disease

Leon J. Thal, M.D., Professor and Chairman of Neurosciences and Principal Investigator of the Alzheimer’s Disease Cooperative Study, University of California, Los Angeles

The United States has undergone a demographic shift since the early 1900’s with more people living longer. With this shift, the medical challenges facing the elderly population have become more prominent. Alzheimer’s disease (AD) is currently recognized as the most significant disease of aging.

As people age, some develop a syndrome broadly termed “dementia,” which is a deterioration in intellectual functioning coupled with memory deficits. AD can be attributed to about two-thirds of patients with dementia. Common symptoms of AD include memory loss and impairment in delayed recall, and disturbances of language, judgment, vision, and behavior. The exact cause of this disease remains unknown, though amyloid (a fatty-looking substance found at high levels in the brain of those with the disease) neurotoxicity is the leading hypothesis. There are several risk factors for AD including age, family history, and genetics.
Little was known about AD prior to 1970, and there still are not effective treatments to slow progression, delay onset, or prevent the disease.

It will be important to use epidemiological studies as observational tools for generating clues about AD, and then it will be important to use clinical trials that are properly designed and controlled to make discoveries that will deepen our understanding of the disease. Dr. Thal suggests that, ultimately, advancement in AD treatments will require greater investment in basic research, a reworking of HIPAA to eliminate barriers, improvements in the clinical trials infrastructure, and a faster system to award grants.

According to Dr. Thal, progress in treating this disease has been hampered by:

- A lack of good animal models;
- Difficulties in determining the actual cause of the disease;
- Inadequate research funding;
- An inadequate clinical trials infrastructure;
- Lengthy regulatory approval procedures; and
- A lack of innovative research by the pharmaceutical industry.
Epilepsy

Introduction by Warren Lammert, Founder and Chief Investment Officer, Granite Point Capital and Co-Founder, The Epilepsy Project and epilepsy.com

Warren Lammert explained that epilepsy afflicts 2.3 million people in the United States, with children and seniors experiencing the highest rates of new seizure onset. Given that this disease disproportionately affects seniors (vs. other adults), the incidence of epilepsy is expected to increase as the population ages. Epilepsy, defined as having two or more unprovoked seizures, is really a spectrum of diseases, and the need to find better treatments is paramount because it is a chronic disease that, like diabetes, transforms the way people live their entire lives. There have been about nine new therapies developed over the course of the last decade for epilepsy. But, currently available medications do not relieve seizures for 20 to 40 percent of those with the disease.

From an industry perspective, epilepsy is a moderately-sized market, so it is not a prime focus for the pharmaceutical industry. For this reason, the Epilepsy Project has focused its efforts on improving the process of translation and commercialization of new therapies and is particularly interested in the economics of the process. The Epilepsy Project has a strong scientific advisory board with a broad range of research development and clinical expertise, and works collaboratively with the other groups working in epilepsy.

Warren Lammert shares the impact epilepsy has had on his family – and what steps are needed to cure the disease.
The Epilepsy Project is focusing on a defined series of “platform” or infrastructure investments that will reduce costs and enhance returns for all new epilepsy therapies. Projects include:

- The development of an epilepsy brain tissue bank;
- The development and maintenance of epilepsy.com – a critical information resource for patients, clinicians, and researchers; and
- Regulatory advocacy to persuade the FDA to change its current approval process for new epilepsy treatments in order to make the new therapies available for monotherapy use rather than restricting approval (and therefore reimbursement and de facto use) solely to combination therapies.

Commentary by Jacqueline French, M.D., Professor of Neurology,
The Neurological Institute, University of Pennsylvania School of Medicine and Co-Director, Penn Epilepsy Center

Dr. Jacqueline French provided an overview of the impact of the disease on patients. There is no biomarker or any kind of marker to indicate the future course of seizures. To move the research agenda forward, she proposed the creation of an epilepsy clinical trial consortium network that would be run by academic centers rather than the pharmaceutical industry. Patient information would be accrued longitudinally in databases, which could lead to a better understanding of the course of epilepsy over time. This consortium would benefit from the successes and lessons learned from other similar clinical trial consortia organized for diseases like Parkinson’s and asthma.

Commentary by Orrin Devinsky, M.D., Professor of Neurology,
Neurosurgery and Psychiatry, New York University (NYU) Medical Center and Director, NYU Comprehensive Epilepsy Center

Dr. Orrin Devinsky spoke about the Epilepsy Phenome/Genome Project, which is a comprehensive, large-scale repository of clinical, diagnostic, therapeutic, and genetic data information on patients with epilepsy. The goal of this project is to provide new insights into the mechanisms responsible for intractable epilepsy. The hope is to identify specific genetic, molecular, and neuropharmacological pathways that contribute to an individual’s risk for developing the disease in order to target new treatments more effectively and possibly even to create preventative measures.
What Color Is Your Diet?
Using Nutrition to Accelerate Medical Solutions

David Heber, M.D., Ph.D., FACP, FACN, Professor of Medicine and Director, Center for Human Nutrition, University of California, Los Angeles

Dr. David Heber discussed how to accelerate medical solutions with nutrition. Studies done at the PCF concluded that somewhere between 10 to 20 percent of an individual’s condition was related to genes, what their ancestors had given them. Likewise, 80 to 90 percent of their condition was reliant on how they conducted their life. Nutrition plays an important yet overlooked role in human health and disease prevention and offers an opportunity to reduce healthcare costs by focusing on prevention.

Plants have great potential for health and for inexpensively accelerating medical solutions for diseases such as cancer. Cancer is a devastating and expensive disease worldwide, yet we now know that chemicals derived from fruits and vegetables can stimulate DNA and repair enzymes damaged by cancer, actually preventing the expression of certain genes.

As Dr. Heber points out in his diet book, What Color is Your Diet?, nutrition is the key to improving American’s health and ability to combat debilitating diseases. This country might spend up to 20 percent of our gross domestic product on medical costs next year but proper nutrition gives us the opportunity to reduce healthcare costs by simply focusing on prevention and what we eat.

“So what's wrong with our diet? In a nutshell, too much fat, too much sugar, too much starch, and not enough protein. Protein is associated with lower body weight, better satiety, and lower incidences of heart disease. Plants are a good source of essential nutrients such as protein but too many of the food items available in this country have been bred for marketing through visual appeal and have lost much of their nutritional value and true color. There are 150,000 edible plant species on earth yet only 150 to 200 are eaten; only 20 are popular in the United States. A fruit or vegetable’s color is an indicator of the chemical compounds it contains. Glucosinolates found in vegetables like broccoli are green and work in your liver. Lutein, the yellow-green substance, goes to the back of your eye in the retina where the most ultraviolet light is concentrated. Anthocyanidins are purple and work on mental function.”
WHAT COLOR IS YOUR DIET?

- 10-20% of health is genetic
- 80-90% Behavioral & Environmental
- NCI used to regard nutritional advice as deceptive & useless — times have changed.

Herbs have great potential
- Growing green tea in California
- Originally a medicinal herb — a tonic — beginning of tea consumption
- Many herbs boast health benefits — let's reinforce the benefits.
- Food industry sells taste, cost & convenience — not health.

Need to pay attention to REBALANCING our diets...
- Need to look at 860 varieties of plants
- In the next 10 years, Type II diabetes will be a major contributor to heart disease.

Only 300 species eaten — 25 commonly in U.S.
- Red to Green
- Color vision correlates
- Colors indicate phytochemicals
- Eating fruits & vegetables makes up a different part.

- Many people have consumed the whole of COLOR.
- Every color means something — go heed the whole family of compounds — the chemicals!
- Better continue — similar study produced negative results because of compound CONTRAST.

When you see the COLOR— it means...
Session 6
The Fast Track to Faster Cures
Immediate Opportunities for Accelerating Medical Initiatives

Stunning success has been achieved in treating heart disease and strokes over the past decades. Future advances in molecular biology, genetics, immunology, and neuroscience could produce similar gains against a wide range of deadly diseases. This session of the retreat focused on what audacious, meaningful, and immediate opportunities could be taken to realize those gains. Participants were asked to consider three categories: research resources, research organizations, and the research environment, and then propose initiatives and opportunities to impact each of these areas.

Greg Simon described the three categories in detail:

- **Research resources**, i.e., databases, information, training of people who are conducting the research, and communicating the results of the research;

- **Research organizations**, i.e., NIH, private institutions such as the Howard Hughes Medical Institute, and academic medical centers; and

- **Research environment**, i.e., the relevant financial and economic policies affecting research, FDA regulations, and social implications/context.

Kauffman Foundation executive Bob Litan listens intently as Liz Connell, legislative aide to U.S. Senator Ted Stevens, offers perspective on NIH funding and collaborations.
Research Resources

The first issue discussed was the need to increase the number of people participating in clinical trials. Suggestions included:

- Survey those who have been most successful at clinical trial enrollment and disseminate those practices as a model.
- Have FasterCures convene a workshop on clinical trials to gather thought leaders to discuss the issues inherent in conducting research overseas and the research protections that would be needed. Additionally, the impacts on drug pricing in the United States could be covered.
- Increase the internationalization of trials and the full integration of the pharmaceutical and device industries.

The second issue discussed was the need for greater availability of research data. Suggestions included:

- Increase the use of electronic medical records and standardized data capture, as this would increase data availability and dissemination of data.
- Require, by law, that all medical records be electronic.
- Develop better data standards and enforce them.
- Create a community of interest around the data access question comprised of patients, doctors, and the research community.
- Increase patient awareness of why medical information is valuable and necessary to research.
- Ensure that appropriate safeguards for privacy exist or it will be difficult to persuade patients to make their medical information available.
Research Organizations

The discussion about opportunities to transform research organizations largely revolved around identifying problems and making modifications to NIH. Problems with the status quo at NIH that were identified included:

- There are some, including those on Capitol Hill, who believe NIH has developed a conservative culture that tries to protect the way it does business, and could act as a barrier to change;
- NIH has no “Manhattan Project-like” initiatives and no mechanisms to support such large-scale coordinated efforts;
- Various disease-oriented advocacy organizations and the institutes themselves are unwilling to give up the resources necessary to support large trans-NIH projects.

Suggestions for addressing these barriers included:

- Change the way NIH funds and conducts research in order to merge various research disciplines, such as biology, molecular imaging, and nanotechnology, in mounting major coordinated attacks on diseases such as cancer;
- Implement a DARPA-like project funding and management model as a potential stimulus for innovation;
- Transform NIH into a more goal-oriented research center by mandating the establishment of goals within the NIH intramural research program that would focus on markers of progress within specific disease areas.

“The model that NIH has chosen for itself is the model of a university. NIH wants to have the very best scientists, winners of Nobel prizes, and run on the same incentive system as a university, where each individual investigator is rewarded for his or her research prowess, working in a small group of people. So it’s no different than the external scientific community. If you want an audacious goal, it would be to redevelop NIH as a goal-oriented research center.”

David Baltimore
Research Environment

The final part of the discussion focused on what opportunities exist to promote faster cures in the research environment. The discussion centered on changes to the regulatory environment and suggestions included:

- Minimize the negative impact of HIPAA on the conduct of research;
- Centralize IRB policies and approval processes for multi-site trials. One possible solution could be a central IRB that is authorized by the Office for the Protection of Human Subjects to handle multi-site trials; and
- Reform the FDA approval process by examining the incentive/risk ratio.

Wrap-Up Discussion

Greg Simon led a wrap-up session where the conference participants were given the opportunity to suggest action items that were not discussed during the previous sessions. Suggestions included:

- Examine how to deliver new treatments and cures to patients faster;
- Create a training program for medical journalists;
- Set up contacts between U.S. industry and foreign research institutes;
- Get NCI more involved in the early stages of the development pipeline;
- Get NCI more involved in tissue banks and information technology companies; and
- Create an advisory committee for medical database initiatives.

Greg Simon moderates a late afternoon session on the lawn of Mike and Lori Milken’s Tahoe home about audacious goal-setting for making the complex machinery of medicine work faster.
Closing Session

Greg Simon’s Comments

The FasterCures “Attack” at Lake Tahoe was the beginning of a discussion among the FasterCures Board, our staff, special guests from various disciplines and members of the Tahoe Group. The discussion did not end when the meeting did. Already, new collaborations, new opportunities and new ideas are flowing like the streams that fill the Lake. The challenges we face are immense, but so is our commitment.

The future of the Tahoe Group is in the making. We at FasterCures need the advice, experience and energy of the group to propel our work farther and faster. We need the diverse perspectives and talents to help us see the future we are trying to create. And we need the encouragement, support and participation of people whose own lives demonstrate the great difference personal commitment and effort can make in the world.

Let me answer the hardest question we always get: Why do we think we can succeed? Because we are determined to do so – and because people like the ones who attended our first annual retreat are here to help us.
Thank You!

FasterCures / The Center for Accelerating Medical Solutions would like to thank the following people for making the first annual FasterCures Tahoe Retreat possible:

The Lake Tahoe community for their hospitality, including
- The generous residents who hosted Retreat participants
- Lori and Mike Milken for allowing their home to be used throughout the weekend
- Camille and Larry Ruvo for an entertaining evening at Shakespeare Ranch, and
- Sierra Nevada College and president Ben Solomon for providing meeting space

FasterCures Board members for their participation: Dr. Ernest Bates, Dr. David Baltimore, Nancy Brinker, Michael Klowden, Dr. Gerald Levey, Shmuel Meitar, Dr. Richard Merkin, and Mike Milken

Chefs Beth Ginsberg, Blake Machamer, and Gus Rivas for healthy sustenance

Graphics facilitator Tom Benthin for capturing the proceedings

Staff of the Milken Institute for their support in organizing the event

And, last but not least,

FasterCures thanks each person who traveled short and long distances to attend the Retreat, enthusiastically participated, and committed to making a difference in the urgent mission to save lives by saving time.
To catch breaking news in the medical solutions arena, sign up for the FasterCures SmartBrief – a free twice-weekly e-mail – at www.fastercures.org/smartbrief