Venture Philanthropy Strategies to Support Translational Research: Workshop Summary

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FORUM ON NEUROSCIENCE AND NERVOUS SYSTEM DISORDERS

VENTURE PHILANTHROPY STRATEGIES TO SUPPORT TRANSLATIONAL RESEARCH

WORKSHOP SUMMARY

Sarah Hanson, Lori Nadig, and Bruce Altevogt, *Rapporteurs*

Forum on Neuroscience and Nervous System Disorders

Board on Health Sciences Policy

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Willing is not enough; we must do."
—Goethe

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*IOM planning committees are solely responsible for organizing the workshop, identifying topics, and choosing speakers. The responsibility for the published workshop summary rests with the workshop rapporteurs and the institution.

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This report has been reviewed in draft form by individuals chosen for their diverse perspectives and technical expertise, in accordance with procedures approved by the National Research Council's Report Review Committee. The purpose of this independent review is to provide candid and critical comments that will assist the institution in making its published report as sound as possible and to ensure that the report meets institutional standards for objectivity, evidence, and responsiveness to the study charge. The review comments and draft manuscript remain confidential to protect the integrity of the deliberative process. We wish to thank the following individuals for their review of this report:

Sophia Colamarino, Autism Speaks
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Although the reviewers listed above have provided many constructive comments and suggestions, they did not see the final draft of the report before its release. The review of this report was overseen by **Sharon B. Murphy,** scholar-in-residence, Institute of Medicine, who was responsible for making certain that an independent examination of this report was carried out in accordance with institutional procedures and that all review comments were carefully considered. Responsibility for the final content of this report rests entirely with the authoring committee and the institution.

Contents

Introduction[1]

The purpose of the workshop was to convene representatives from a variety of voluntary health organizations with experience in establishing and developing translational research programs supported by venture philanthropy strategies. Participants provided valuable insight into a wide range of considerations for other voluntary health organizations who are seeking to start or develop their translational research programs. Discussions centered on best practices and lessons learned in order to improve efficiency and effectiveness in translational research.

Voluntary health organizations—that is, nonprofit charitable organizations, patient advocacy groups, and foundations—have a long-standing history of providing support to those suffering from disease. Historically, this giving fell under a few key areas: buying and distributing medicine; providing food, care, and shelter to those in need; offering education; and delivering prophylactic equipment to target areas, such as mosquito nets in malaria-stricken regions. In addition, many voluntary health organizations have supported basic research, helping to seed the development of innovative treatment ideas that commercial pharmaceutical and biotech companies can advance into the clinic and develop into novel therapies. Recently, some voluntary health organizations began to shift from providing care, educational resources, and funding research grants to now

[1]The planning committee's role was limited to planning the workshop, and the workshop summary has been prepared by the workshop rapporteurs as a factual summary of what occurred at the workshop.

supporting the earliest stages of drug development and engaging in translational research.

On October 3, 2008, a wide range of participants, from voluntary health organizations to academic investigators to industry representatives, gathered at the Arnold and Mabel Beckman Center of the National Academies of Sciences and Engineering in Irvine, California, for a workshop titled "Venture Philanthropy Strategies Used by Patient Organizations to Support Translational Research" (the workshop). Participants were selected from a variety of backgrounds and were asked to discuss and share their own experiences and lessons learned as their organizations moved into a translational research program supported through venture philanthropy strategies.

Workshop chair Timothy Coetzee, executive director of Fast Forward of the National Multiple Sclerosis Society, noted that embracing venture philanthropy does not mean turning away from original and basic science research. In explaining why the dynamics are shifting, he reiterated that while supporting scientific discovery is still important, it is also important to develop new funding models that bring products into the clinic. Workshop participants focused on the how and why of developing such new funding models to bring products and, most important, hope to patients.

VENTURE PHILANTHROPY

An increasing number of voluntary health organizations are looking at venture philanthropy as a critical way to advance their mission of helping patients and working to cure disease. The concept of "venture philanthropy" stems from venture capitalism, which invests money from various third-party sources in typically high-risk areas. For example, in medical research, adopting a venture philanthropy approach entails operating within the translational space, working through one's funding and strategic leadership to help draw discoveries out of the academic sector and into the hands of parties with the ability to commercialize new therapies. A venture philanthropy strategy is unique in that its mission is aligned with philanthropic goals and outcomes—namely, new therapies and cures for diseases—and whose efforts are supported primarily by individuals and foundations whose urgency for such cures is great. The standard approach to research funding has not demonstrated sufficient results, and venture philanthropy represents a new model by which

patient-relevant outcomes may be more quickly achieved. In the case of this workshop, many of the strategies discussed are focused on becoming more directly involved with the drug development process while relying on the generosity of foundations, wealthy individuals, and other sources of nongovernmental funding.

The Need and Risks

The reality is that drug development—even for large, relatively well-funded diseases—is a very slow process. Joyce Nelson, president and chief executive officer of the National Multiple Sclerosis Society, shared some sobering statistics during the workshop's opening remarks. Prior to 1993 there were essentially no effective treatments for multiple sclerosis. In the past 15 years, only six partially effective treatments have been approved, and a cure seems far away. Across the disease landscape in any given year, the FDA approves only 12 to 16 truly novel therapies (new molecular entities). For every drug that receives FDA approval, 10,000 fail.

Workshop participants discussed the new reality in industry-driven drug development. As drug development costs have risen, due primarily to the high failure rate present in every stage of drug development, an increasing number of companies are reducing or halting investments in risky, early-stage product candidates in favor of later-stage opportunities with a greater likelihood of success. At the same time, the "blockbuster" mentality of many pharmaceutical companies—which is directly tied to this same high cost of drug development—has limited the development of drugs for smaller or more challenging diseases. The risks are simply too high for the private sector to tackle alone. Recognition of this new reality is what is driving voluntary health organizations into venture philanthropy, said Coetzee.

Michael J. Fox put it this way (as quoted by Nelson): "The tough truth is that the drug development funding system is broken where risk is highest." Venture philanthropists, therefore, need to get in early, where the risks are highest, and fund the research that will attract the capital markets later. "As patient advocates, we exist to make a difference in the lives of our constituents," said Nelson. "Venture philanthropy is a tool for advancing research that will transform lives. It is the human return, not the financial return, upon which we must focus."

TRANSLATIONAL RESEARCH

The workshop focused specifically on using venture philanthropy strategies to support "translational research," a tricky phase in the drug development process that bridges the gap between the halls of academia and commercially funded clinical trials. Translational research is a broad term used to describe the process of translating the basic biology of a disease into real-world therapeutics in the lives of patients. Because the term is so broad, it can be difficult to bring organizational focus to the shared challenges and opportunities that a full-spectrum approach to venture philanthropy can bring.

Dennis Choi, vice president of Academic Health Affairs at Emory University, discussed a commonly agreed upon schematic of translational research that begins at the identification and validation of targets for research and ends with clinical research trials (Figure I-1).

A Precompetitive Funding Model

Several associations and federal agencies, such as voluntary health organizations, the National Science Foundation, the Department of Defense, and the National Institutes of Health (NIH), fund the best ideas in academia research, the most promising of which are picked up by industry. The knowledge and technologies from this research may initially fall under the precompetitive space, an area where companies, sponsors, and developers can collaborate on research and development in a space that does not confer a competitive advantage to any individual partner (IOM, 2008). However, the current linear model for conducting and funding drug development research is problematic, said Choi, because it puts different types of organizations working at discrete points of the value chain with little or no overlap (Figure I-2).

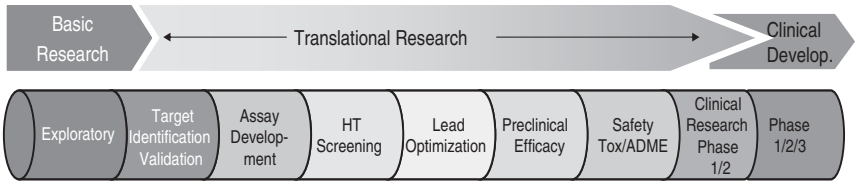

FIGURE I-1 Translational research model.
SOURCE: Insel, 2008.

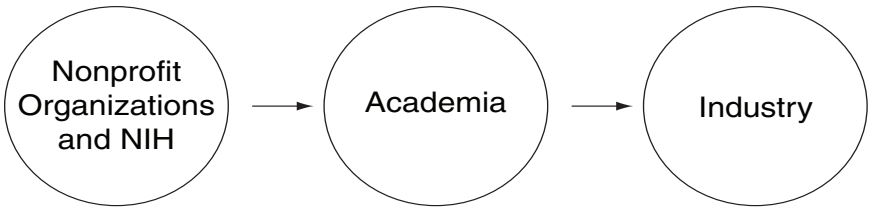

FIGURE I-2 Precompetitive funding model, linear process.
SOURCE: Choi, 2008.

Choi described some of the challenges related to his own institution's work on central nervous system diseases: the multitude of expressed targets, the difficulty of crossing the blood–brain barrier, the limitations of current animal models, the length of treatment required, and the difficulties of clinical trials. Because the central nervous system is so complex and provides difficulties for drug development, the current linear model where different players work independently from each other simply does not work in his opinion.

Choi illustrated a different model where in a precompetitive space, each entity works in partnership to advance science in an environment of collaboration (Figure I-3). Even within industry, the individual small-scale players would band together to fund core research that will benefit everyone over the long term.

Choi believes this model is both possible and recognized as necessary by the stakeholders involved. This move toward a new model, where voluntary health organizations, academia, and private industry work together for the benefit of each, framed a fundamental question: How can we bridge this gap?

Fortunately, there are some examples for how it has already shown success. The Alzheimer's Disease Neuroimaging Initiative (ADNI) was a partnership launched a few years ago by the National Institute on Aging and includes representation from several NIH institutes, foundations, and industry (ADNI, 2007). The Foundation for the NIH (FNIH) is another example and includes a cohort from the NIH, Food and Drug Administration, and the Pharmaceutical Research and Manufacturers of America (FNIH, 2008). These, among other collaborations, illustrate how various groups are working together in a precompetitive space to advance the understanding of a disease or group of conditions.

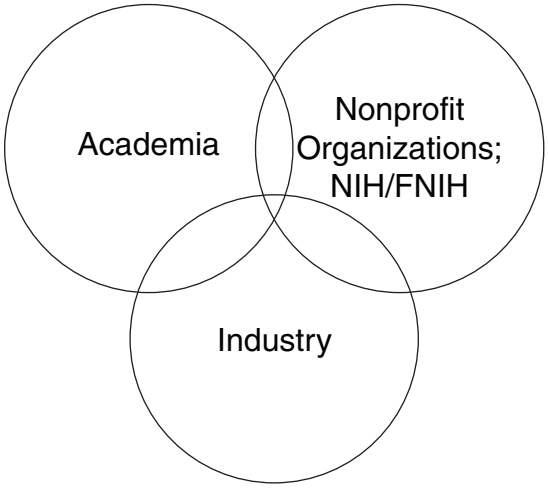

FIGURE I-3 Precompetitive funding model, partnership focused.
SOURCE: Choi, 2008.

ABOUT THE FORUM AND THE WORKSHOP

The Forum on Neuroscience and Nervous System Disorders (the Forum) was established by the Institute of Medicine (IOM) in 2005 to bring together the public and private sectors, among other key stakeholders, to discuss issues of mutual interest and concern on topics of common and critical importance, particularly those issues and topics that stimulate partnerships to accelerate understanding and treatment of nervous system disorders. Recognizing that there was much that voluntary health organizations could learn from each other's efforts, an independent planning committee of the Forum organized a workshop to discuss this new model for financing medical research.

The workshop convened a group of key stakeholders and experts representing a variety of voluntary health organizations involved in venture philanthropy–supported translational research and tasked them with identifying and sharing the best practices used, and lessons learned, in order to improve efficiency and effectiveness in translational research.[2] The breadth of experience was the best asset. Some of the invited indi-

[2]To download presentations or listen to audio archives, please visit http://www.iom.edu/CMS/3740/35684/57121/57131.aspx.

viduals represent organizations that have been running venture philanthropy programs for years and are involved in clinical trials; others are still in the discovery or development stages. Some are on the funding side of the equation; others are the recipients. Recognizing that the topic was broad, the planning committee structured the workshop around a single hypothetical case study. Workshop participants were furnished with a basic scenario to guide presentations and discussions (see Appendix B for workshop agenda). In the case study, a hypothetical voluntary health organization, the Colten Foundation, is seeking advice on establishing a translational research program for the development of a novel drug, biomarker, or diagnostic tool. The Colten Foundation is attempting to anticipate, and get ahead of, the challenges it may encounter. It is inquiring how other organizations that have faced these challenges have tackled them as it seeks to establish best practices, solicit general advice, and, it is hoped, position itself for success.

The workshop was co-chaired by Timothy Coetzee, executive director of Fast Forward of the National Multiple Sclerosis Society, and William Thies, vice president for medical and scientific relations at the Alzheimer's Association. In this workshop summary, key pieces of advice and experiences are attributed to one individual, or when multiple parties were involved with fashioning or honing a single idea or insight, the idea is attributed to the key parties involved. The summary follows the flow of the workshop, mirroring how the day progressed from general discussions to more specific topics, and section headings are organized to reflect shifts in topic and focus.

This workshop summary does not put forth specific recommendations or consensus statements by the IOM or the Forum, but rather serves as a mechanism for various stakeholders to share their experiences and advice.

A number of issues were addressed over the course of the day, and, while not exhaustive, some of the major themes that were supported by several participants are emphasized below. First, the importance of knowing the organization's strengths, weaknesses, and goals will help to reinforce when an organization can and should become a central player and leader in the field, when partnership with others is most valuable, and how to prioritize areas of consideration that are in keeping with the organization's mandate. Second, the significance of adaptability in major processes (grant making, partnerships, reorganizations, and failures) will keep the organization from plateauing or faltering from its mission. Finally, the organization's role as a liaison to and spokesperson for the pa-

tient community is invaluable. What the organization provides for patients (e.g., education, advocacy, support, leadership) is just as important as what the patients provide the organization—be open and prepared to really listen to the patient community. A complete list of key points can be found at the end of each chapter.

1

Getting Started in Translational Research

The workshop was structured around five sessions, each focusing on a set of specific topics that the hypothetical voluntary health organization, the Colten Foundation, will need to consider as it establishes or develops its translational research program. In this chapter, the Colten Foundation is seeking advice on establishing and maintaining a productive and efficient translational research program. It is particularly interested in the criteria considered and the decision matrix used by other voluntary health organizations in their decisions to begin a research program for the development of a novel drug, biomarker, or diagnostic tool. Participants began by offering some initial considerations when getting started in translational research.

Creating novel mechanisms to support translational research is a large step, and for many voluntary health organizations it is a leap into the unknown. Several voluntary health organizations have spent decades in the core business of raising money and supporting basic research, providing direct patient care, supplying capital for facilities, or making small-scale individual academic grants. Louis DeGennaro, chief scientific officer of the Leukemia & Lymphoma Society, encapsulated some of the critical crossroad questions a foundation needs to ask when it begins thinking about new approaches to support a translational research program: what is the state of the art, the science, and the medicine?

Illustrating these points, DeGennaro discussed how 10 years ago, the Cystic Fibrosis Foundation was ready to begin supporting a translational research program. The science was at a stage where legitimate product development could occur. The foundation seized on the opportunity, and today the cystic fibrosis community has seen a rise of venture philanthropy–backed translational research.

The move toward translational research implies a critical change in how a voluntary health organization approaches its mandate, and there can be no one-size-fits-all approach. For the Leukemia & Lymphoma Society, for instance, the current state of science and medicine in blood cancers puts them somewhere in the middle. For example, there is a great deal of basic science that remains to be done, so the society continues to fund core academic research programs. Basic research is important because it is necessary to lay a strong foundation in the basic understanding of the disease; only then can researchers effectively identify targets. The Leukemia & Lymphoma Society, for which there are real opportunities to drive therapies out of the lab and into the clinic, has been increasingly investing in translational research.

For the society, that meant conducting a formal and extensive review of the existing research portfolio, explained DeGennaro. By looking at the research projects it was already funding through the lens of pharmaceutical drug development and discovery, it found that more than 10 percent of its already funded projects were actually in the development stage. This showed that the state of the science and medicine in the society's disease space was advanced enough for substantial clinical development work to begin. It had the kinds of targets, assays, and biomarkers ready and available to put a focused translational development project in place, said DeGennaro.

Through the discussions at the workshop, many participants agreed that there are key factors that need to back up the decision to fund translational research. Richard Insel, executive vice president of research for the Juvenile Diabetes Research Foundation, offered four key points to consider:

- Know your disease
- Know yourself
- Know your partners
- Identify your goal

KNOW YOUR DISEASE

To know where to begin and what needs to be funded, voluntary health organizations need a strong understanding of their target diseases and conditions—a map of their diseases. What is really known about the disease? What is unknown yet needs to be known in order to facilitate

drug development? Celia Dominguez, vice president of chemistry for CHDI Management, Inc., noted that most degenerative diseases, even monogenic ones such as Huntington's disease, have yet to be scientifically described at a sufficient level of detail to even enable the drug discovery process.

Katie Hood, president and chief executive officer of The Michael J. Fox Foundation for Parkinson's Research, suggested that a simple tool, a checklist of knowns and unknowns, was extraordinarily helpful for the foundation to organize thinking around a disease. The list allowed the foundation to examine its disease space and focus its funding on checking off the unknowns as efficiently as possible. The objective of this methodical approach is to reach a tipping point where there is enough knowledge about the disease and basic research in place to create attractive opportunities for the private sector to step in, continue research and development, and drive therapies into the clinic.

This is fundamentally where voluntary health organizations can add the most value because they can operate outside the bottom-line focus of a publicly traded life sciences company. "We should be addressing things that other people don't want to address because the risk is simply too high," Hood said. She put forth some of the questions a voluntary health organization should ask about its disease space on the basis of her foundation's work with Parkinson's disease (Box 1-1). The answers to these questions will expose the critical area where the lack of funds and lack of knowledge meet.

For any one disease, this assessment of the drug development pipeline will require examining the state of multiple targets and lead hypothesis. The development of this combined knowledge—the knowns and unknowns of a disease and close analysis of the drug development pipeline—has turned out to be a unique asset in the field for The Michael J. Fox Foundation. In addition, it is important to find out where the money is being spent, where it is not, and why. Over time, developing ways to share this analysis and assessment with the broader field, constantly communicating new developments as they occur, becomes an important contribution in the grant funding that voluntary health organizations provide.

BOX 1-1
Guiding Questions for Setting Up a Translational Research Program

- Has the community congregated around a common set of hypotheses or mechanisms?
- Is there a short list of high potential targets?
- Are successful, functional, and predictable animal models in place?
- Are there tissue banks and shared resources?
- Do biomarkers exist?
- Do clinical trial resources exist?
- How advanced is diagnosis? (For Parkinson's disease, the only definitive diagnosis is via autopsy, creating significant problems for clinical studies.)
- How long does it take to measure the success or failure of a clinical therapy? (For Parkinson's disease, the answer can be 5 years when evaluating a neuroprotective agent.)
- Is there a clear path to and through the Food and Drug Administration?

SOURCE: Hood, 2008.

Biomarkers

A major focus of translational research is on bringing potential targets and candidates from the laboratory through development and to the patient, but this research also requires a focus on the development of tools like biomarkers to facilitate and speed this process. Workshop participants agreed that early biomarker development could greatly aid in shortening the time for industry to step in and fund research. Insel explained that for type 1 diabetes, the focus of the Juvenile Diabetes Research Foundation, clinical trials last as long as 2 years, as opposed to a 3-month trial for psoriasis or a rheumatoid arthritis trial that takes 6 months. The identification of a reliable biomarker that could be used as a clinical trial end point could shorten trial times, increasing the attractiveness of diabetes to commercial research. Biomarkers may have analogous roles as diagnostics in clinical practice, for example, to enable individualized medicine.

Hood agreed and expressed that Parkinson's disease drug development is slowed not just because markers do not exist to definitively diagnose Parkinson's disease or measure its progression, but because biomarkers of a drug's effectiveness are also inadequate. For a voluntary health organization involved in translational research, investment in biomarkers is as important as investment in promising new therapeutics.

For example, research in all types of biomarkers—diagnostic, disease progression, disease subtype, and drug activity or placement biomarkers—can be extremely valuable and improve therapeutic discovery.

KNOW YOURSELF

A recurring theme of the workshop was that a voluntary health organization must explore its strengths, weaknesses, resources, and culture in order to pursue any new venture, especially something as dramatic as a move into translational research supported through venture philanthropy. In many cases now, there is a unique role for voluntary health organizations because their value lies not only in funding, but also in their leadership, convening power, ability to bring people from different groups into the same room, and ability to articulate an overarching research agenda for the field. "I think it is critical to be very clear what you see as your mission, what your role is, what your niche is, and what your risk tolerance is," said Insel. Developing a translational research program requires many things from a voluntary health organization: commitment, expectation management, the readiness to constantly examine and reexamine progress and risk tolerance, and the willingness to change when things are not working. If an organization's culture is resistant to this process, it may not be the right time to move into funding translational research.

Joyce Nelson of the National Multiple Sclerosis (MS) Society warned that you must be prepared to face resistance. "Adapting to a new model means change, and change disrupts the status quo," she explained. "That was particularly true for [the National MS Society] as we have been exclusively funding university research for some 60 years." The idea of using charitable donations to fund private industry was a big cultural shift.

Commitment

Commitment from organizational leadership is key to support the program by hiring and developing the internal staff needed to bring the "big ideas" into reality, said workshop chair Timothy Coetzee. Beyond internal staff, there is also the commitment to educating other stakeholders and managing expectations. DeGennaro explained that "Volun-

teers, board members, patients, and stakeholders get very excited when you pull the trigger on a biotechnology company alliance that triggers the initiation of a clinical trial immediately." His own group was lucky enough to have the resources to fund two such trials in the 12 months prior to the workshop. Such a ramp up in work beyond basic science turned heads. "There is a huge expectation built there that needs to be managed because the vast majority of such studies will be failures," he explained.

Expectations

One of the biggest challenges that many voluntary health organizations face is managing expectations, too much hype, and too much overpromise, explained Insel. Translational research is a difficult endeavor, and many times, he says, the public does not realize that. Because of this you need to educate stakeholders about what to expect. "You want hope, not hype." In addition, it is important to have a clear line of sight to your goal and metrics to evaluate your success. This is made easier, Insel said, when everyone within the organization and all the partners are on the same page as far as time lines and milestones.

Risk Tolerance

The last critical area for self-awareness is the definition of risk. What level of risk is the Colten Foundation comfortable with? How does the foundation define risk? What level of risk are its donors comfortable with? As Insel explained, there are ways to build safety nets to manage risks, particularly with research facilities, by building in milestones and reviewing projects as they progress. It is a common business practice, but not one as common in academic science as it is in, say, commercial building construction. Once milestones are set and a review process is established, a sponsoring organization has the tools needed to halt a project if it goes off track. But Insel was quick to point out that the assumption of risk was where venture philanthropy had its strongest role. "If we don't take on risk as a foundation," he explained, "nobody else is going to take it on. So really the obligation is on us to take on risk."

Each of these areas—establishing commitment, managing expectations, and analyzing risk tolerance—needs to be evaluated not just within

the walls of the organization, but in the broader base of donors, patients, and advisers. Keeping these sometimes disparate groups of stakeholders motivated, committed, and involved is critical for success.

The Importance of Opinion

Sometimes, the most difficult piece of self-knowledge is simply knowing what you want. Hood related how The Michael J. Fox Foundation came to understand that it was important for the foundation to actually have a vocal opinion about the priorities in the field of Parkinson's disease research. The foundation began with the idea that it wanted to fund research faster, put fewer constraints on researchers, and facilitate quicker applications and action on promising lines of investigation.

As it convened review committees and distributed funding quickly, the foundation realized that it did not know what other Parkinson's disease groups, the government, or biotech and industry were doing in the field. "What we were doing felt random," she recalled. "We didn't know the landscape of what other funders were doing in our space. So how did we know that what we were doing was actually needed?" The foundation launched a formal analysis to assess the research and funding landscape. Then, on the basis of these findings, it developed a plan for where its resources should be invested.

Many organizations have voiced similar concerns and eventually have come to realize that opinions about what should be funded vary with who is in the room at any given meeting. Many have found that those in the room have different viewpoints and a different stake in the process, and therefore different needs. As a result it has become clear that a voluntary health organization needs to have its own opinion about where priorities are in this field. This underlines the significance of positioning the organization as a central player in its field. Voluntary health organizations are in a unique role in that they can provide an overview of the state of the research, in addition to providing insight into and education for the patient community. An organization's leadership and culture determine where the organization will tread, so it is important that everyone is on board.

KNOW YOUR PARTNERS

Voluntary health organizations are not the only players in the battle against disease. The NIH, academia, and the private sector bring strength to the table, but nobody can fund the entire spectrum from basic science through to clinical approval. As Dennis Choi of Emory University highlighted, forming partnerships with academia, industry, government, and other voluntary health organizations is necessary in order to take a particular therapy all the way from idea to cure.

Partnership management was a common theme throughout the workshop. The importance of cooperation and collaboration was emphasized, as well as the challenges that inevitably arise from such relationships. The message to the Colten Foundation was this: know your strengths and weaknesses going into this program, and, as you enter into agreements and collaborations with partners, learn their strengths and weaknesses too. It is important to go into partnerships with the goal of leveraging the partners' strengths and being sure to understand their motivation, suggested Dominguez.

Working with the Private Sector

Hood shared that in the first 4 years of the foundation's existence, there was little meaningful communication with partners. "When you start thinking about your goal being a cure … the fact that we hadn't engaged industry in any of our discussions meant that we were really getting only one side of the equation." Engaging early in a dialogue with industry was one of the lessons learned. Try to understand what the needs are and what it will take from a scientific standpoint to get industry partners further engaged. This information could help to prioritize your grant process.

Working with the private sector, however, implies an entirely new set of "know your partner" responsibilities that is beyond what most voluntary health organizations are used to considering. As Insel shared, financial and organizational stability issues can come back to haunt you. While most companies are well funded and viable, there are no guarantees, and Insel explained that the foundation has had its share of problems with unstable companies. He suggested that for the Colten Foundation, the way to protect the foundation was to enter into partnerships with rigorous contractual requirements covering these issues and

provide recompense when interruptions occur. (Specific legal issues are addressed later in this summary.)

Public–private operational differences can contribute to misunderstandings, delays, and duplication of efforts. The worlds of academia and industry are different in several key ways, said Insel. For example, understanding the difference between clinical research and clinical development is important. Oftentimes data that have been collected from clinical research trials are not applicable to company efforts; consequently, either the trial has to be completely redone or the data have to be reanalyzed. It will save a lot of time and effort to address this up front, Insel said.

IDENTIFY YOUR GOAL

As was discussed previously, knowing your target diseases and conditions, or having a map of your disease, is significantly helpful. A voluntary health organization can then assess how translatable the findings from basic research are to industry partners, which will help gauge the disease's attractiveness and the likelihood of a meaningful partnership.

There exists a breadth of tasks that live under the umbrella of translational research, and Insel highlighted the importance that venture philanthropy may offer in bridging not only the funding gap, but other important gaps as well. Voluntary health organizations using venture philanthropy can help engage researchers and guide their efforts toward a "sweet spot," Insel said, where proof-of-concept trials begin as quickly as possible in order to remove as much risk as possible from the broad gap between basic research and clinical development. Ultimately, voluntary health organizations need to approach the process of translational research the same way pharmaceutical companies do. "The game is about creating a pipeline," said Insel. He warned against betting on a single product; a better approach is to recognize that some level of failure is inevitable. The key is to figure out how to move each stage forward, keeping a line of sight to a product, while at the same time realizing that one organization cannot do everything. The trick, according to Cynthia Joyce of the Spinal Muscular Atrophy Foundation, is to do small things really well.

There are many important gaps that a voluntary health organization may choose to focus on. For example, any target suggested by basic research must also be treatable with clinical therapies (pharmaceutical or

otherwise). Oftentimes, what works in an academic lab is not suited for the high-throughput world of drug development. In his experience, Insel said that the Juvenile Diabetes Research Foundation learned that many of the assays that had been developed in the academic community had to be reformatted for compatibility with high-throughput screens. Thus, a voluntary health organization may choose to focus on validation.

Another area, mentioned previously, is the identification of a reliable biomarker that could be used as a clinical trial end point that could shorten trial times, increasing the attractiveness of a given disease to commercial research. Biomarker research is an area where venture philanthropy is critical because it is often hard to get other partners committed to this area, said Hood.

A focus on the achievable is especially important for a young organization. Hood commented that The Michael J. Fox Foundation was founded with the idea that the amount of money it was going to be able to raise was going to be limited, so focusing its efforts and articulating why the funds raised were going to move the needle on developing a therapy for Parkinson's disease was a key concept.

PREPARATION NEVER STOPS

Workshop participants articulated the need to constantly reexamine one's organization to learn from mistakes, improve procedures, and adapt. The most successful voluntary health organizations are highly introspective and willing to change as needed. Hood explained her approach: "We are constantly iterating on what we do. We look very carefully at what we think worked, what we think didn't work, and who we need to talk to to get better at what we're trying to do."

Coetzee summarized some key points at the end of the session (Box 1-2). As the Colten Foundation works toward developing a translational research program, understanding what is known and unknown about its disease, understanding its culture, and learning about its potential partners can help it decide if now is the time to move into translational research. Likewise, the foundation needs to be prepared to educate its constituents, even as it reexamines its efforts to invest in the best and most promising projects.

BOX 1-2
Key Points: Getting Started in Translational Research

- A large part of the initiation phase is a thorough analysis of the state of the science and medicine.
- Do not rely on a single matrix for decision making; the process is unique to an organization in many ways. It is better to develop a process for coming to a decision.
- Understand your target diseases and conditions; this will help you in your decision making, prioritizing, and communications.
- Make a list of knowns and unknowns, and focus funding on addressing and overcoming the unknowns as efficiently as possible. Once there is sufficient basic research in place, it becomes a more attractive opportunity for the private sector to step in, continue research and development, and drive therapies into the clinic.
- Examine and understand the drug development pipeline (i.e., where money is being spent and why).
- Embrace the organization's role as a key source of leadership, convening power, and research agenda setting.
- Identify where critical unmet needs exist and establish a strategic plan for addressing them.
- Be flexible when it comes time to examine and reexamine progress and goals. Keep this in mind when you encounter failure.
- Be prepared to face resistance when you attempt to disrupt the status quo.
- Encourage and foster commitment from organizational leadership to hire and develop the necessary staff who will drive ideas into reality.
- Communicate well with stakeholders and manage expectations.
- Devise metrics to evaluate your success and keep your goals in line.
- Define your organization's priorities early on, and understand that it is in the nature of a venture philanthropy program to assume a certain amount of risk that other nonventure philanthropy programs will shy away from.
- Recognize that collaboration is key; leverage your and others' strengths.
- Get industry involved early through formal and informal collaborations and dialogue.

2

Models for Building a Translational Research Program

In session two, the hypothetical voluntary health organization, the Colten Foundation, has decided to go forward with a translational research program and is interested in hearing about models of organization and leadership structure. How can a program be organized to ensure appropriate expertise and leadership are available? What financial models are effective and efficient? Workshop participants offered insight and provided details of some model for addressing these questions.

After the decision has been made to utilize venture philanthropy strategies to support a translational research program, an organization naturally begins to consider looking at structural models that have worked in the past for other, similar organizations. Questions about how best to recruit leadership, attract bright investigators into your disease space, and fund sustainable research arise, and much can be gleaned from the experiences and best practices of other voluntary health organizations.

APPROACHING SCIENTIFIC DISCOVERY

Jonathan Simons, president and chief executive officer of the Prostate Cancer Foundation, put forth two different models for thinking about how to approach scientific discovery that might be applied to voluntary

health organizations that are just starting to set up their translational research program. In the "Lewis and Clark expedition" model for scientific discoveries, there is a very clear objective. Drawing from the Lewis and Clark expedition, President Jefferson issued a mandate to explore, map, and document the Missouri River and move westward, discovering the most useful waterways from which to engage in commerce from ocean to ocean. There was no time line. The expedition reported back both successes and failures. It had the latest state-of-the-art tools, as well as the funding and support needed not just to test a given hypothesis (that a particular path of navigation was viable) but to engage in pure exploration, both generating and testing hypotheses as it progressed. Applying this expeditionary model to a translational research program would imply a diverse, well-funded, flexible, and independent effort.

As an alternative to this expeditionary model, Simons outlined the "NASA" model. NASA's Gemini and Mercury programs were concerned primarily with testing and retesting every system and every procedure required to meet the long-term goal before actually mounting the Apollo missions and moving on to the eventual moon landing. Each aspect of the long-term mission was broken down into individual missions, each conducted independently. Rather than invest in broad, sweeping research and letting each incremental discovery redefine the mission, the NASA approach breaks the problem down into discrete units and explores them independently, increasing the likelihood of making the final "moon shot" successful. Simons sees value in such an approach for translational research. "[It would be better to have] 20 Gemini missions in Parkinson's disease where you learn an enormous amount than it would be to have three failed, large-scale, basically phase 3 trials."

LEADERSHIP

In every successful grand challenge, there is always *someone* in charge who has not only the knowledge and vision to guide a program, but also the leadership abilities to get things done, explained Linda Van Eldik, professor in the Department of Cell and Molecular Biology at Northwestern University Feinberg School of Medicine. Leadership must include people who can be active participants in that vision. One of the biggest leadership challenges for many translational research programs is cultural—specifically, knowing how to navigate the differences in academic and corporate leadership culture.

Traditionally, the leadership model has been that academia and academic advisers drive the agenda, but workshop participants believe this model is changing. Cynthia Joyce of the Spinal Muscular Atrophy Foundation explained that for a patient advocacy organization, soliciting advice and guidance from academia and thought leaders and driving research programs is not unusual. What has become increasingly clear, but sometimes less obvious, is that there is no one paid to spend 100 percent of his or her time following a problem from start to finish. This creates a leadership gap, where foundations need to step in and act as the focal point for the research, said Joyce. This is especially true of rare diseases, where the volume of work is relatively small, she added. Increasingly, voluntary health organizations are doing more than just writing checks by presenting research results at scientific meetings if they believe they have the scientific expertise and the broad, constant-attention view that the research needs. Becoming more involved and actively leading the scientific process is an important role for a voluntary health organization. This, of course, amplifies the importance of having a strong, active scientific advisory board, according to Joyce.

Hosting scientific meetings is another way that voluntary health organizations are establishing themselves as experts in many fields of endeavor. They are the conveners and information brokers and can provide an overview of the research landscape. Katie Hood of The Michael J. Fox Foundation for Parkinson's Research agreed and shared that increasingly the foundation receives inquiries from investors, companies, and researchers who want to know who they can talk to or should be talking to in this space. They are often confused about or do not know how to navigate the different players and rapid information flow, and what they want to know is if there is a go-to person who can help point them in the right direction. Increasingly, this "person" is the foundation, she said.

Future Leaders: Addressing the Pipeline Issue

Richard Nakamura, deputy director of the National Institute of Mental Health, believes that there is a desperate need to attract more individuals into research to create not just a year's worth of research, but a sustained effort that could ensure the pipeline is full. "There's no doubt that the science right now is great," he commented, "but our ability to train new scientists and keep them in science has been dropping precipitously. We're losing a whole generation." Rae Silver, professor of natu-

ral and physical sciences at Columbia University, echoed his concern and expressed that science careers are becoming increasingly less attractive. "There are so many threats around everything that you do, it's increasingly difficult to get research funding, so scientific careers don't seem to be that interesting and exciting," she said.

Participants agreed that the best way to attract new researchers to a specific disease or area of interest was to recruit. The term "recruit" is used to refer to several ways that voluntary health organizations are attempting to attract high-quality researchers to their disease space. By investing directly in researchers and funding their careers, a voluntary health organization such as the Colten Foundation could ensure a pipeline of talent for years to come. The Prostate Cancer Foundation, for example, is directly funding the careers of key researchers, said Simons.

Joyce explained that in the case of the Spinal Muscular Atrophy Foundation, the disease is relatively understudied, so it was important to first raise awareness of the disease in order to recruit individuals to work on core research and identify new areas for discovery. The foundation invested in recruiting scientists to the field, and Joyce explained that part of this outreach has been through the contract research organizations conducting its fee-for-service research. By increasing its presence in the contract research organization industry, the foundation has increased awareness of the disease overall and the foundation in particular, which will keep new scientists involved with the disease.

Similarly, when The Michael J. Fox Foundation started recruiting scientists to its staff research team, it focused on identifying talented young researchers—frequently senior postdocs—who had not yet fully embarked on an academic career. Hood believes that the decision to hire younger scientists who had not yet committed to a single path of research was essential for the foundation to develop an open-minded approach to the field.

FUNDING MODELS

Once strong leadership is in place, decisions on how to fund and how involved to be with various programs can be made with the voluntary health organization's long-term goals in mind. Workshop participants spoke about a balance between an organization's need for accountability and researchers' need for freedom. Van Eldik put it this way:

> Historically, investigators would apply for a grant from a foundation or another funding organization and they'd get the grant, and there would be no oversight, no accountability for what happened with that money. All of that is changing. Because now, if you are going to try to develop something further on, especially for drug discovery, you need to have a set of milestones that you're accountable for. There's a balance between having too restrictive a milestone that doesn't allow any flexibility in the work to be able to take advantage of some of this serendipity that might come up, and having no accountability.

Funding through a grant is a big incentive for academic investigators, concluded Van Eldik, and often investigators work with foundations because of it, but it is also important to recognize that not every project requires the same level of management and interaction. For example, The Michael J. Fox Foundation's Rapid Response Program for early-stage, hypothesis-driven work is very different from the foundation's other programs. Funding decisions are made extraordinarily quickly, within 6 weeks of receiving an application. There are no milestones. There are no mandatory deliverables. In effect, there is no management to be done, because the nature of the science does not require it.

In other funding programs, The Michael J. Fox Foundation is highly involved in the process. Hood said that in the case of a drug development, multistage grant, the foundation is significantly involved, sitting at the table during decisions. This level of intense involvement is not always welcome and can in fact be problematic. Hood explained that because of this policy of involvement, the foundation found that some researchers would simply walk away rather than put up with what was seen as intrusive management, especially when, like the Colten Foundation, The Michael J. Fox Foundation was new to venture philanthropy.

Ultimately, Joyce maintained, the Colten Foundation should seek to be a source of not just capital, but smart capital. This essentially means being direct and proactive about funding decisions, focusing on working within partnerships that are productive and funding the best opportunities.

The Base Model: Program Grants

While there is no single model of funding research, the most basic is the simple grant. For example, the Spinal Muscular Atrophy Foundation's program grants can include basic, translational, and clinical research. Based on the project at hand, deliverables and milestones are established to ensure that all of the parties involved talk to one another. But these basic grants are themselves changing, and sometimes traditional academic contracts begin to look almost exactly like industry ones, said Joyce.

The Alternate Model: Fee-for-Service

When there are research needs that simply are not attractive to either academia or industry, a voluntary health organization has an opportunity to bridge this gap by paying for the work to be done on a simple fee-for-service basis. At the Spinal Muscular Atrophy Foundation, one such gap was the development of a viable screen assay, Enzyme-Linked Immunosorbent Assay (ELISA) for spinal muscular atrophy, a project that had languished in the academic community. "The best way to do it is with a contract research organization," said Joyce. Fee-for-service contracts such as the Spinal Muscular Atrophy Foundation ELISA project are one of the most unique ways for a foundation like Colten to make a difference—by directly targeting a "dead zone" in the disease space and applying capital.

The discussion provided a good list of major issues covered in session two; these are summarized below (Box 2-1).

BOX 2-1
Key Points: Models for Building a Translational Research Program

- Sometimes you need to spend time and energy testing various hypotheses in a methodical way.
- Strong leadership with a broad vision and set of goals is essential.
- If you have the scientific expertise and a broad view, then do not be afraid to lead the scientific research.
- Expand the workforce pipeline by investing in researchers and their careers.
- Try to strike a balance between researcher freedom and foundation accountability.
- Be flexible in how you approach each project—not every project needs the same level of interaction and management.

3

Legal, Accounting, and Process Issues

In session three, the hypothetical voluntary health organization, the Colten Foundation, has a leadership team in place and is beginning to think about challenges it may face in terms of appropriate accounting and legal practices, as well as other process-related issues. It is interested in learning about what other organizations have already done. Workshop participants offered key points of consideration and specific lessons learned, as well as provided legal and accounting models from their own experiences.

Setting up any large organization is challenging and complex, and a large-scale translational research program is no exception. Some of the issues a new program will face are fairly routine—for example, accounting and financial reporting for a large nonprofit, while nontrivial, is a documented process based on widely accepted principles. The primary task is hiring competent staff and outside accounting support. But there are challenges that are unique to the translational research field and that may require the assistance of knowledgeable legal counsel with real domain expertise. Workshop presenters discussed a wide range of issues and proposed a series of questions the Colten Foundation should consider as it works to build the bones of its organization.

SOCIAL ACCOUNTING

Proper financial processes must be established to ensure that a voluntary health organization is being fiscally responsible and fulfilling the requirements of tax law. But in the voluntary health organization world, many workshop participants also believed that some level of social accounting was necessary to ensure that a group is supporting its charter, advancing the group's broader mission, and providing a positive impact on its constituents. In order to achieve these ends, an organization should develop mechanisms to assess and measure the progress and impact it is having on the field.

While a typical venture capital fund will try to get a return of 30 percent to 40 percent each year, Peter Heinecke, chief business officer of Experimed Bioscience, suggested that voluntary health organizations would probably be satisfied with much less than that. "A venture philanthropy fund has a double bottom line: one line is still return, but the other line is the social good that you are advancing," he explained. These two bottom lines exist in tandem because while the Internal Revenue Service requires one, a voluntary health organization's donors and constituents require both.

It is important for an organization to have reasonable near-term expectations for its social bottom line. For most voluntary health organizations, the long-term goal is to cure or eliminate disease. However, other achievable goals are nearer term and help to measure investment and medical success; therefore, resetting expectations to include shorter-term definitions of success while not losing sight of the longer-term ones is smart, Heinecke suggested. Those definitions will be dependent on the target of each grant or project. For organizations funding a small start-up, success might be finding animal models that provide enough proof of concept to help them attract new private-sector investment. For a later-stage program, the definition could be the conclusion of a successful clinical trial. Heinecke also insisted that even in failure an organization must take the time to understand why it failed and commit to making data and information available to others.

Carol Mimura's Intellectual Property & Industry Research Alliance Department at the University of California, Berkeley, also uses a double bottom line and agrees with defining success in short-term blocks. The bottom line in terms of social impact is lives saved and medical costs reduced, she explained, but these are not easy metrics to collect. The eventual social impact of an action might be decades removed. The ad-

vice to the Colten Foundation is to ensure due diligence, but also work to create and communicate its own nonfinancial definitions of success.

LEGAL ISSUES

The legal activities of any voluntary health organization are designed to do two things: protect the organization and provide a legal framework under which the actual work gets done. Operating in a product-driven (and sometimes profit-driven) world raises a host of contractual issues that do not exist in the nonprofit community. Setting up successful legal agreements in these situations requires extensive communication and due diligence between the parties involved, as well as an understanding of the issues that may arise. The more questions that are answered prior to entering into a contract, the more assured an organization can be that it will be successful and protected in the case of failure.

Return on Investment

The Colten Foundation will need to make a fundamental decision about its approach to investment returns: does it demand a return on investment from funded partners? Many foundations feel they should receive a return on the investment they made in academic research, said Kenneth Schaner, a lawyer in private practice with extensive venture philanthropy experience with the Cystic Fibrosis Foundation among others. However, Schaner shared that while he has put it in many agreements, the truth is that they have not received many returns.

One problem with returns is simply the time frame. Typically, the time between when a grant is given and when a marketable invention is out of the lab and on the market is quite long. Often, there are previous or subsequent grants (and grantors) on the pathway from research to product. In the case of a successful, marketable product, there is little debate that a funding organization should get a portion of the proceeds, says Schaner, but what portion?

In Schaner's experience the answer is frequently that any return should bear the same relationship as the grant had to the total cost of developing the product. This determination introduces complexities of its own, and many organizations simply forgo any attempt to recapture

revenue from academic contracts. Yet, he maintained, others keep it in because one contract may be unique from the next.

Process Considerations

More than any other part of the workshop, the discussion of legal and contract issues was one of simply highlighting a variety of areas that participants had dealt with in the past. The following subsections outline some of the questions and considerations that the Colten Foundation may encounter as it moves forward with its translational research program. Where available, the rapporteur has included relevant experiences shared by participants.

Accountability

Most voluntary health organizations live under clear mandates to use the organization's resources to pursue specific goals. It is critical that an organization be able to assure donors that the money they give will be used on the projects they are being asked to fund. Likewise, if the Colten Foundation makes an investment, how will it know that its money is being used as intended? One way to do this is to include an interruption, or "diligent commercialization," clause. These clauses state that if a contracted partner fails to continue research in a field for a period of some consecutive number of days, then the contract is terminated and the organization gets exclusive rights to the results of the research, explained Schaner.

Of course, the intent is never to invoke these clauses. Most foundations will not be well served by engaging in legal battles over intellectual property (IP) rights (see Chapter 4 for more information on IP). Even if a foundation succeeds in gaining control of IP, assigning that IP to a new research team or hiring a contract research organization to complete the work has both implicit and explicit costs.

Mimura's group at the University of California, Berkeley, takes a slightly different approach. If the licensee of an IP right is not performing, one option is to reserve the right to reduce an exclusive license to nonexclusivity so it can be relicensed, she explained. With an exclusive license, there is sometimes a mandatory sublicensing clause, as with Mimura's office. Such tactics would give the Colten Foundation substan-

tial flexibility without invoking draconian terminations. If the foundation becomes aware of unanticipated uses of a particular IP, either the licensee has to pursue it or the foundation can seek additional partners. In this way, no undeveloped new niche will go unaddressed, said Mimura. In her experience, companies rarely object to such terms because many see it as free market research.

Core Structure

Should the Colten Foundation establish a separate entity to award grants? A separate entity can be convenient, but it is not strictly necessary. Workshop participants emphasized that indemnification from grantees and insurance is still needed to protect the foundation.

Domain

Will the Colten Foundation assert rights to any application of the research it funds? If the research yields results for a disease not in the Colten Foundation's disease space, how does the foundation want to treat those revenues?

Due Diligence

How detailed does the assessment of a particular grant need to be? What are the assumptions in the due diligence process?

Intellectual Property

Will the Colten Foundation have the rights to the new invention through some form of option, especially if the awardee does not use it after a particular time? What role will it take in owning, maintaining, and enforcing IP rights?

Involvement Level

How passive will the Colten Foundation be in its grants? Does the foundation want to take a more active role? Schaner cautioned that the more active an organization is, the higher the possibility of liabilities.

Milestones

With near-term, easily identifiable, and unambiguous milestones, the Colten Foundation's contracts will be less difficult to evaluate and enforce. Linda Van Eldik of Northwestern University Feinberg School of Medicine thinks most voluntary health organizations have made good progress in striking the balance between excessive micromanagement and loss of control. Clear goals, milestones, and continuous open communication are in place with several successful academic, industry, and foundation partnerships, she explained.

Royalties

Traditionally, investing in research through a grant with an industry partner is structured to provide return royalty payments to the voluntary health organization. Some organizations also invest in direct equity or purchase debt from a target company. All three avenues have implicationns that can create both complexities and opportunities. For example, if the Colten Foundation gives a grant in return for royalties, accounting rules treat those grants as program expenditures. But if the Colten Foundation invests through equity, it would be treated as an investment. That has an impact on fund-raising ratios and, depending on the Colten Foundation's fund-raising profile, could be important.

If the Colten Foundation will require royalties as the condition of a grant, it needs to consider the terms of the royalty agreement. For instance, will royalties be capped, limiting the return back to the foundation? In capping the return, explained Heinecke, "the company is not worried that it has given you too large a royalty rate, hampering its ability to license the drug or raise venture funding later on." The issue of capping a royalty return can be argued from the opposite side too. As Schaner suggested, "when the investment is large, when we're talking

about taking the risk that no one else is going to take, then a traditional royalty is appropriate."

Raw Return

Even an established royalty stream can be difficult to value. Equity, on the other hand, is in most cases priced frequently in venture capital valuations, so the Colten Foundation would have a sense of what its investments were worth. Equity generally tends to produce a return more quickly than a royalty structure; in addition, many biotech companies are merged or go public well before they make any money, said Heinecke.

Termination

What happens if the Colten Foundation does not like the path a line of research is taking? Or if other developments prove more promising, leading to a particular grant's irrelevance? A voluntary health organization cannot write in the contract that it can terminate the contract in one month, for example, because the contracted company has to hire people to do the research, commented Schaner. Ultimately, the organization has to strike a balance between assuring the contracted company that it can hire the appropriate staff and having the right itself to get out of the program.

Tool Sharing

It is usually in the interest of a voluntary health organization that has developed various research and diagnostic tools to make them widely accessible to researchers in the field. Providing access to new tools is one key way to advance an organization's "second bottom line," discussed previously in this list. Issues involving the licensing of developed property should be addressed up front when Colten is entering into preclinical research contracts.

Cynthia Joyce of the Spinal Muscular Atrophy Foundation shared an evocative example. To continue a line of research for spinal muscular atrophy, the foundation needed access to an animal model. While one existed, it lived behind the walls of an academic investigator who was not going to be part of the research. The foundation had to obtain li-

censes to the mice and put them in a facility where they could confirm that they were characterized and standardized so that the foundation could compare drug studies, recalled Joyce. The process took 3 years. Some voluntary health organizations now deal with the issue of sharing newly developed research tools up front in the contracting, before they are even developed. This could save years of repetition later due to an outdated contract. The Spinal Muscular Atrophy Foundation has taken things a step further, actually prenegotiating material transfer agreements and sublicenses if it is required for research tools.

Case Study: Fast Forward, LLC

A thorough, process-driven funding decision matrix that involves extensive due diligence on the legal, financial, and scientific aspects of a potential opportunity is extraordinarily helpful, according to Timothy Coetzee of Fast Forward. Before a contract is signed, a grant written, or a check disbursed, the Colten Foundation needs to have a process in place. Andrea Tobias explained the process that Fast Forward, LLC, uses for funding.

Fast Forward is the dedicated venture philanthropy group inside the National Multiple Sclerosis Society. The Fast Forward process for making grants and investments was modeled after the Wellcome Trust's process. In the Fast Forward model, the foundation gives loans or grants that are ultimately converted into either series A or series B stock in the particular biotech company.

The objective is not solely to get a return on investments, but also to invest in the best life science technologies. Fast Forward took Wellcome Trust's application process and remodeled it. The application process is designed to be onerous and to act as a significant gate so that application proposals that reach the review stages are in order and can move quickly to the next steps, explained Tobias.

The second step is far more rigorous. Fast Forward's core due diligence process is identical to that of a traditional venture capitalist. A team of experts descends on the target company and produces an exhaustive due diligence book covering the science, technology, clinical protocols, IP, and management team at the target company. This book also includes any other due diligence reports from the other venture capitalists that are looking at the opportunity with Fast Forward. This package is

passed on to a scientific and business advisory committee for review and comment.

Next, a conference call is held with the company, and the company makes a presentation. By the end of the conference call, a decision is made on whether or not the company will move on to the next step, which is presentation to the foundation's board. At the board meeting, the proposal is presented very much like a venture partner would present it to his partnership. A case is made, including the recommendations from the scientific and business advisory committee review and core investment justifications. A vote is taken, and the investment is made or not made. Fundamentally, this is the venture capital process applied in a different domain.

Key messages from session three are captured in the summary box below (Box 3-1).

BOX 3-1
Key Points: Legal, Accounting, and Process Issues

- Knowledgeable legal counsel and accounting support with real domain expertise is critical.
- Social accounting is necessary to ensure that a patient group is supporting its charter and advancing the group's broader mission.
- Set short-term milestones in addition to longer-terms ones.
- Devise a plan of accountability for how to keep your partnership in good standing.
- Recognize that sometimes the more active your organization is, the more liabilities you have.
- Address issues involving licensing of developed property as you enter into a preclinical contract.

4

Partnerships, Data Sharing, and Intellectual Property

In session four, the hypothetical voluntary health organization, the Colten Foundation, wants to expand its portfolio of contributors and partners in order to develop an effective research program. This has led to many questions around data sharing, partnerships, and intellectual property. What should the Colten Foundation consider as it builds its program? Workshop participants discussed the answers to this question in the context of best practices and lessons learned.

A fundamental task for voluntary health organizations supporting a translational research program is creating and managing networks and partnerships. The importance of collaboration and coordination between academics, the National Institutes of Health (NIH), voluntary health organizations, and the private sector in the work to find cures and treatments could not have been underscored more during the meeting. Everyone in the medical and philanthropic community relies on formal and informal partnerships. There are significant challenges to forming these partnerships, and understanding how to navigate these challenges is just as important as having resources to invest.

PARTNERSHIPS

Academic Partnerships

Often there is a gap between the investigation of disease mechanisms in academia and drug discovery in a corporate pharmaceutical world, said Linda Van Eldik, professor in the Department of Cell and Molecular Biology at Northwestern University Feinberg School of Medicine. She believes that there needs to be open communication around the fundamentals of what an industry partner is looking for in the drug discovery process from the research done in an academic setting. When the final recipient of the research is a company that will ultimately need to mass produce a drug, it is essential to get academics on board with the drug discovery mission and process, Van Eldik explained.

A challenge in partnering with academia is cultural, particularly surrounding the tenure process. If modern science requires unfettered collaboration and information flow, this creates a real barrier to investigator and institutional buy-in. Van Eldik believes that the issue can begin to be addressed by getting the institutions behind the translational research process, which is not always valued in the tenure track culture of academia. A major question that arises is how to do "team science" and how individual investigators get credit for team science. What is required is creativity and compromise from all parties. "Academics do *want* to do this," she said. "They *want* to translate their basic science discoveries. It's just [a matter of] figuring out the best way to partner with the appropriate people."

Partnership with the National Institutes of Health

Any discussion about funding for medical science inevitably turns to the NIH. Partnering with the NIH gives a voluntary health organization's efforts credibility in the research process that might be missing if the agenda were driven solely by the organization, according to the workshop participants. Further, voluntary health organizations and the NIH are not competing entities but are instead tremendously complementary.

Many expressed that the NIH is essential to biomedical research, and while not traditionally focused on funding applied science, this is changing with relation to new drug development. Consequently, there exists an opportunity for voluntary health organizations to address this gap and

fund more applied science, according to Katie Hood, president and chief executive officer of The Michael J. Fox Foundation for Parkinson's Research.

A natural way for a new voluntary health organization to enter the research process is to participate in collaborative projects with the NIH on early-stage research. The interaction has the chance of being mutually advantageous, as the NIH may also benefit from the work of organizations. When collaborating, these entities support each other: The NIH provides critical research funding in areas such as biomarkers, while voluntary health organizations can help guide the NIH agenda by sharing the results of translational research.

Building Credibility Through Partnerships

A voluntary health organization is often the most obvious, most easily accessible expert in a disease space. In this role of disease expert, an organization such as the Colten Foundation can bring tremendous value to a partnership with a for-profit company. Peter Heinecke, chief business officer of Experimed Bioscience, Inc., suggested that if an organization does its due diligence and decides to invest in a particular area or company, venture capitalists take note of that and are more likely to also invest. A voluntary health organization probably already knows far more about a disease area than anybody in the venture capital firm does or is going to be able to learn.

But that role as disease expert is just one bargaining chip in what is always a negotiation. There might be many restrictions that an organization is going to want to put on the company, said Heinecke, adding that the challenge is to discover the common ground in any given deal. "In the end you have to come up with a package that is balanced, that overall is an attractive option for the company."

Liaison to the Patient Community

Tricia Brooks, managing director for alliance development at BIO, believes that some voluntary health organizations are particularly good at what is often a missing link in the process: working with the medical community and patients once a treatment is commercialized. As we move toward personalized medicine and look at these better-targeted

therapies, we are going to need someone to play that role in education, Brooks said.

An example is Herceptin, a treatment for breast cancer that is used in highly personalized medicine. Breast cancer patients with the HER2 gene can take Herceptin to reduce the odds of recurrent cancer. Many patients with breast cancer do not have the HER2 gene, yet they demand Herceptin from their physicians because they believe it to be a blanket therapy. It is a classic case where better education is needed, and a case where breast cancer foundations are working to deliver the simple message. This type of patient education will become more common as more targeted treatments are discovered for various diseases, especially as treatments cross boundaries between pharmaceutical manufacturers or involve cocktail treatments.

The direct connection that voluntary health organizations have to patients is perhaps the most valuable contribution an organization can make and enables awareness of the disease from a patient perspective. "You bring expertise that no company can walk in with," Brooks advised. A patient organization knows from its patient community that often the incremental outcome measurements are far different to a patient than they may be to a researcher, and it is important that the foundation bring this message out.

Limiting Risk in Partnerships

One major role in a partnership for a voluntary health organization is to remove risk from relationships. Venture philanthropists step into the translational research gap, funding research that makes future industry investment more attractive and, in fact, possible. The voluntary health organization's role in connecting the dots between academia and the clinic can go beyond simply awarding grants. Margaret Anderson, chief operating officer of FasterCures, listed just a few of the ways voluntary health organizations "de-risk" translational research (Box 4-1). Each of these activities removes a level of risk from the process, making it easier for industry to be involved sooner in the process and helping ensure that funding flows into academic research as efficiently as possible. In many instances, it makes sense to simply approach industry and ask what the organization can do to de-risk their involvement in the process.

BOX 4-1
Limiting Risk in Translational Research Opportunities

- Develop preclinical tools that can be used by other partners.
- Target research to support translation from basic science to clinical trials.
- Create funding mechanisms that bring in industry investment.
- Directly manage academic science.
- Provide access to a motivated patient community.
- Provide access to biospecimens.
- Research new indications for existing drugs.
- Develop high-throughput screening of promising compounds.
- Provide access to scientific, often in-house expertise.
- Act as a powerful advocate with the Food and Drug Administration.

SOURCE: Anderson, 2008.

DATA SHARING

Sharing data successes or failures runs against a common cultural problem, the silo effect. "Some data and resources may as well not exist because you cannot access them," said Sharon Hesterlee, vice president of translational research at the Muscular Dystrophy Association. Workshop participants discussed the silo phenomenon throughout the day in many guises: animal models behind laboratory doors, valuable data from failed research endeavors, narrowed funding sources, even varied research methodology. Even in things such as end point development, what you quickly find out is that you have academic groups doing it different ways, said Hesterlee. Sometimes this difference makes data incomparable from one trial to the next; thus, aggregate data can be very hard to examine.

Data sharing is critical to advancing toward cures by avoiding repeat efforts between voluntary health organizations focused on the same disease space, said Anderson. A common theme at the workshop was the need for the hypothetical *Journal of Failure* or *Journal of Negative Results*, a place where data from failed research could be collected, shared, and discussed. Understanding why something failed can sometimes yield important information that helps researchers move an idea forward or suggest entirely new directions for research.

Data and products from studies should be centralized and shared, advocated Anderson. There are many different tools to do this, and with the advent of new information technologies, data sharing can be substantially improved. But Anderson was also quick to point out that these technolo-

gies are not free, nor is the labor required to document and disseminate information. Keeping that efficiency in mind, a few workshop participants shared how they disseminate information.

Most organizations use meetings as a basic way to disseminate information and share data, whether at formal annual meetings or more frequent brown-bag meetings. FasterCures, for example, has a loose affiliation of groups called TRAIN (The Redstone Acceleration and Innovation Network) groups where disparate groups come together throughout the year to share information and innovations. The Prostate Cancer Foundation's annual meeting involves all of the foundation's funded scientists meeting with alumni and outsiders. For two-and-a-half days, they discuss the current state of prostate cancer research, brainstorming and sharing ideas, successes, and failures. Other groups are working to create shared databases.

Hesterlee discussed the Muscular Dystrophy Association's TREAT-NMD patient registry. With a €10 million grant from the European Union, TREAT-NMD put together a massive database of registries from 20 countries in Europe that have agreed to file mandatory data items. The project brings together different stakeholders including patients, scientists, health care professionals, the private sector, and patient groups from many countries to collaborate and discuss what information is most important in the battle against muscular dystrophy. This international registry is going to be hugely valuable to industry and to academic research, claimed Hesterlee. The Muscular Dystrophy Association recognized that this was a valuable system for data sharing and networking and made the strategic decision that any U.S. registry they support needs to have the same core data elements that can be contributed to the European registry. The key to successful data sharing, according to Hesterlee, is to make sure that the voluntary health organization has defined its goals, that all the partners are aligned, and that a good collaborative structure is in place.

Ethics in Data Sharing

The construction of biobanks and patient registries raises not only policy and process issues, but ethical questions. Susan Wolfe, the McKnight Presidential Professor of Law, Medicine, & Public Policy at the University of Minnesota School of Law, agreed that there is a major opportunity to affect the process and the results. Wolfe, a bioethicist,

presented the workshop with some issues already in play at many foundations.

One unresolved issue in data sharing is that of consent. What kind of consent is legally and ethically needed for patient registries and biobanks? For example, if a young child's data are included in a registry, must new consent be sought to keep the child's information in the registry when he or she turns 18? What about patients who were unable to make their own medical decisions when their data were collected and deposited? Do they need to reconsent when they regain that ability?

Once data are in a registry, confidentiality is one of the primary concerns. Wolfe mentioned a study that showed confidentiality to be a bigger problem than first imagined. Even batched genomic data can be reidentified, explained Wolfe, as the study tested the assumption that it is not possible to identify individuals using pooled data (Homer et al., 2008). The findings of the study caused a scramble to improve the registry system from the smallest contributor all the way to the NIH, she said. These kinds of privacy issues feed back into how the Colten Foundation and other organizations manage consent.

Ethically, voluntary health organizations must consider whether data sharing should always be a two-way street. For example, Wolfe posited, what should data banks and biobanks do about return of individual research results and individual findings? She noted that studies asking about this very issue point out that the primary concern patients have is the return of individual research results and individual findings, yet there was no agreement or established practice for doing this. Some voluntary health organizations struggle with what it means to do right by the people who are generously participating.

At the time of the workshop, none of the participants had implemented a data-sharing program that returns data to patients. Lucie Bruijn of the Amyotrophic Lateral Sclerosis Association explained that communication was the key for that organization. When samples are collected for a genome-wide scan, said Bruijn, the consent form indicates that data will not be returned. Because of this clear communication, and the understanding in the patient community about the research goals, there has been little concern that data sharing has been inequitable.

The issue of genetic markers is itself an issue of ethics, not just in the research field, and one that the Colten Foundation may need to contend with as its research progresses from basic science toward therapy. Maria Carrillo, director of medical and scientific relations for the Alzheimer's Association, discussed how this issue has been evolving in the Alz-

heimer's disease community. Recent research has suggested that a specific gene is linked to a greater susceptibility of developing Alzheimer's disease after the age of 55. Consequently, patient studies now routinely collect and analyze data on the gene. But the gene is never disclosed, and there is a reason for that she said: it is an imperfect indicator. The risk of overdisclosure of imperfect science is, in and of itself, an ethical dilemma, said Carrillo.

Maintaining awareness of important ethical issues will be a key factor. Wolfe advocates pulling in bioethicists as a start to help lay out the issues.

MANAGING INTELLECTUAL PROPERTY

Although the issues surrounding ethics often converge on agreed principles such as protect privacy, advance the science, and do no harm, different organizations often have diametrically opposed opinions about intellectual property (IP). Anderson laid out some models that various organizations adopt when approaching IP issues. For some organizations, IP rights are explicitly off limits. These types of voluntary health organizations do not attempt to own IP, because they feel that to do so does not support their stated mission of serving a patient population. At the other end of the spectrum is the voluntary health organization that focuses tremendous energy on acquiring and defending IP because its business model hinges on industry partnership for the development of treatments and cures. Most organizations live in between these extremes, including organizations that do not retain ownership of IP for funded research but that retain some rights if IP owners do not commercialize the property.

How an institution values IP will affect any partnerships it makes. As Heinecke said, a company could say, "As much as I'd like your money and your support, I don't want my IP tied up." A voluntary health organization should look closely at the opportunity and determine what sort of deal can be worked out, he said. Celia Dominguez, vice president of chemistry at CHDI Management, Inc., said CHDI does not maintain IP rights in order to have as much of a collaborative and noncompetitive environment as possible. The organization's philosophy is that to enable downstream development by biotech and pharmaceutical partners, it needs to be able to give them something that actually is of value so they can go ahead and do phase 3 trials and registration if need be.

Case Study: University of California, Berkeley

The Intellectual Property Management Office at the University of California, Berkeley, provides a good model for innovative IP and technology transfer. "Over and over, people are saying the goal should be to expedite translational research, to shorten translational research gaps, to traverse the 'valley of death,'" said Carol Mimura, assistant vice chancellor of Intellectual Property & Industry Research Alliances at the University of California, Berkeley. In 2004, the university set about doing just that, restructuring to align itself explicitly with the goal of accelerating translational research. Viewing itself as an innovation accelerator, the university worked with academia, industry, government, and voluntary health organizations in order to catalyze innovation. "Unless we make use of our network, we can be stranded in many ways, academically and with respect to funding, whereas if we're open-minded to collaboration, we can leverage what we have and what the other entities have many times over," Mimura said.

The university came to view technology transfer not just as a process for obtaining IP rights and licensing them to the commercial sector, but also as a system where IP inflow and outflow are based on individual relationships in a process that can span decades. Mimura feels that the university's goal is social impact, with revenue generation from licensing being secondary. Success, in her opinion, is having the best outcome of research and public–private partnerships, and product development partnerships are the way forward.

Mimura also highlighted the Socially Responsible Licensing Program at the university, where the university is partnering with industry to maximize the humanitarian impact of its research in the developing world. The university is proactively giving away IP rights to companies to commercialize products, explained Mimura; companies are then required to give away or sell those products at cost. The program has had surprising success. The Bill and Melinda Gates Foundation funded $42 million for a low-cost malaria drug, whose price in the developing world will be reduced 10-fold under the agreement. Because the university was able to structure the arrangement from basic science all the way through commercialization, the entire deal was signed in a single day, with a single three-way collaboration agreement and two IP licenses. In a way, that agreement and acceleration is the poster child for accelerated translational research because it dramatically decreased the time it takes to get

from bench to bedside and, subsequently, the drug is expected to be on the market 6 years after signing.

Brooks summarized the message to the Colten Foundation this way:

> We are all part of the same spectrum. If NIH funding doesn't continue … then the pipeline doesn't continue strong. If the FDA doesn't have the expertise … we're not going to get products through. If industry does not remain incentivized, it's only going to slow things down, because industry can go make money someplace else. The people that lose are the patients.

The major points discussed during session four are summarized below (Box 4-2).

BOX 4-2
Key Points: Partnerships, Data Sharing, and Intellectual Property

- Coordinated teamwork, whether formal or informal, and organization are critical.
- Philanthropic organizations and the NIH are not competing entities; rather, they can be tremendously complementary.
- Partnership with the NIH and industry in the research process is critical.
- Clearly lay out the process for drug discovery with all partners so that everyone is on the same page.
- Try to remove as much risk as possible within your partnerships.
- Realize that your role as a true expert in a disease space will provide tremendous value to your partnerships with for-profit companies.
- Find common ground when working with the medical community and patients once a treatment is commercialized.
- Bear in mind that your direct connection with a patient population is an invaluable perspective that a patient group brings to a partnership.
- Be keenly aware of the myriad ethical issues that may arise as your program develops and you begin to venture into areas such as biobanking and registries.
- Consider the merits of public-private partnerships and product development partnerships as you move forward.

5

Communications

In session five, the hypothetical voluntary health organization, the Colten Foundation, is trying to learn more about how best to communicate progress and setbacks with current and potential funders as well as its constituents. It recognizes the delicate balance that is needed between demonstrating progress and limiting false expectations. Workshop participants discussed effective communication strategies for disseminating information on research and other progress to potential funders and constituents.

In large part, the Colten Foundation's successes or failures will rely heavily on communication. It will communicate with donors, staff, media outlets, funding recipients, government agencies, companies, industry groups, and other constituents. How well it succeeds at this communication will have a direct, daily effect on its ability to get things done, and that means it will directly affect the pace of research in its disease space and, ultimately, the well-being of patients. Good communication builds the foundation of trust between a voluntary health organization, its researchers, institutional partners, and constituents—trust that can lead to a powerful, positive reputation for the foundation, which has an impact on funding and effectiveness in the field. It is also a process of education and an exchange of ideas, and a good communication strategy needs to recognize that different audiences need different messages communicated via different media, said Sophia Colamarino, vice president of research at Autism Speaks.

INSTEAD OF MANAGING EXPECTATIONS, LEAD

Throughout the workshop, many participants mentioned the need to keep expectations appropriate. Dan Zenka, vice president of communications at the Prostate Cancer Foundation, suggested leading with a clear and open communication strategy from the beginning so that there is no need to manage expectations because everyone is always on the same page (Box 5-1). Zenka pointed out that this process requires significant management buy-in long before any problems need to be addressed or before a substantial funding or research event occurs. In addition, an organization and its researchers and institutional partners should be in frequent communication. "At the Prostate Cancer Foundation, staff are very close to those that we fund," Zenka said.

ACTING VERSUS REACTING

Cathy Carlson, senior director of research information for the Research and Clinical Programs Department of the National Multiple Sclerosis Society, advised that it was key to have advance notice from either grantees or medical journals about upcoming publications or results so that communication strategies can take advantage of opportunities to showcase readiness and a plan of action. Carlson agreed that communication often becomes most critical when doing damage control or when trying to balance the high expectations for a very promising drug versus

BOX 5-1
Steps to Creating Open Communication

- Show where the foundation wants to go. Announce a program, most likely breaking it into phases, and announce what you believe the end goal is or what breakthrough is sought.
- Define milestones and decision points where each phase of development will end and the next one will begin. Outline possible outcomes, along with problems and anticipated challenges. Define where the go/no-go decisions will be made.
- Give lots of updates along the way. Communicate successes and failures and explain what the failures teach you and where you will go from there.

SOURCE: Zenka, 2008.

the realities of the risks, for example. Zenka advised responding to negative developments and issues quickly, where appropriate. It is important to explain successes and setbacks equally, while pointing out that there are other things in the pipeline, Carlson suggested (Box 5-2).

EDUCATION

Medical research is often as difficult to explain as it is to conduct. One of the most important functions of an organization such as the Colten Foundation is education for the public about the disease, for donors about the research process, and for others concerned about research results, Colamarino stated emphatically. She shared that many times her constituents may not understand the basics of how research is conducted, and it is the voluntary health organization's duty to explain this in the best possible way.

Workshop participants also agreed that many times it is easier to educate donors and nonclinical professionals about programs in general than it is to detail a specific project. "It's easier to sell a program where you have lots of things going on, which you can talk of as a group," explained Carlson. The communication difficulties can actually drive the work itself. Louis DeGennaro, chief scientific officer of the Leukemia & Lymphoma Society, explained that the society bundles its research projects into disease-specific research portfolios of roughly 10 projects per portfolio, with roughly $1 million annual funding in each. "It's allowed some ease in how we respond about advances. You may not have an

BOX 5-2
Proactive Communications

- Where possible, do not give time lines because there are always setbacks.
- Do not assume people have read your existing communications. You cannot confirm that your message gets out to everyone.
- Do not automatically assume that your people can connect the dots. You need to educate potential people by giving them specific talking points to help them get the message out that is both hopeful and puts things into perspective.
- When communications must be reactive, it is important to react quickly.

SOURCE: Carlson, 2008.

advance in each project each year, but you will have at least one advance in each portfolio each year that can be communicated," he explained.

The bundling also becomes a marketing tool, allowing donors to feel like they are contributing to fund a panel of projects rather than a specific project that may or may not yield a positive outcome, said Maria Carrillo, director of medical and scientific relations for the Alzheimer's Association. The Alzheimer's Association bundles its research projects under therapies, genetics, and molecular mechanisms when communicating with constituents and chapter networks. The idea of packaging communication into easily digestible containers aids in communicating results as well.

LISTENING

A voluntary health organization cannot ignore its constituents, Colamarino said. While "listening to your consumer" may seem obvious, in an endeavor as complex as venture philanthropy, in a field as complex as translational research, *how* the voluntary health organization listens is very important. For some workshop participants, this ear to the ground starts with individuals giving presentations around the country and coming face-to-face with constituents. For others, such as the National Multiple Sclerosis Society, it is an information resource center that is mostly centralized and acts as a central call-in center so that the society can track statistics on its constituents' hot-button issues. That kind of direct hot line allows the society to tailor its communications. Listening to the questions donors and constituents ask, Carlson said, will tell an organization what it is not communicating properly and show it the gap that needs to be filled with a different message or the same information presented in a different way.

TOOLS

The good news for voluntary health organizations is that there are more pathways to eyes and ears in their communities than ever before. For example, Sharon Terry, president and chief executive officer of Genetic Alliance, runs an organization that fosters communication between diverse stakeholders working on genetics and health. Genetic Alliance has developed tools to bring together information from foundations and

scientists and make it available across boundaries, pooling data into an extensive, consumer-friendly database that can be accessed through simple web tools.

Another piece of communication for Genetic Alliance is a resource depository, explained Terry, which has templates for material transfer agreements for patents and informed consent forms and information. This is backed up by a tool it calls "Wiki Advocacy," which contains best practices for how to set up research projects, how to bring them through the pipeline, how to do translation, and more. Remarkably, all of this is aggregated by the community. Groups like Genetic Alliance serve as community outposts.

Key points from session five are captured below (Box 5-3).

BOX 5-3
Key Points: Communication Strategies for Patient Organizations

- Communication is multifaceted and should be used as a tool for education and exchange of ideas in addition to getting your organization's message out.
- As a spokesperson for patients in your disease space, you can communicate to scientists and researchers about where therapeutic efforts will have the largest, most immediate effect on real patient welfare.
- Find a way to deliver your organizations key message(s) in the midst of information overload.
- Be proactive by gathering information and preparing communications well in advance.
- Try to always be acting, not reacting, to important news.
- Be forward looking and communicate this view when there are setbacks.
- Keep in mind that sometimes it is preferential to communicate information about programs in general, broad views than to detail a specific project.
- Communication goes both ways; be open to listening and learning from your constituents.

Conclusion

The concept of venture philanthropy is not as new as it sounds. The National Foundation for Infantile Paralysis engaged in venture philanthropy 75 years ago. Supported by donations from ordinary Americans, the foundation funded much of the research that led to the development of the polio vaccine, up to and including the final field tests that demonstrated the vaccine's viability in real-life settings. The foundation succeeded because it had the determination, insight, and energy to pursue an endeavor that neither public institutions nor private companies could pursue on their own. The effort required massive involvement at every stage of science, from basic research through testing and on to commercialization. The program worked because the foundation stepped out of the public–private model for paralysis at the time and directly engaged in bold science.

This model of venture philanthropy was largely lost in recent decades, as nonprofit and government funding increasingly focused on early-stage research, leaving later-stage development and commercialization to the for-profit sector. Today, however, things are changing. Evolving circumstances in the drug development sector have created new gaps in the research process that voluntary health organizations are uniquely suited (and increasingly willing) to fill.

THE TRANSLATIONAL RESEARCH GAP

The modern drug development process has become extraordinarily costly and risky. It has been estimated that only 1 of every 10,000 new drug candidates succeeds, and moving from initial discovery to full com-

mercialization is very expensive (Nelson, 2008). As a result, private industry has focused less on earlier-stage development and instead on later-stage research, where the chances of success are higher. Consequently, a gap in the process of translating basic research into commercially viable product candidates exists.

This has an effect on translational research at the academic level as well. Novel discoveries made by clinical researchers about the nature and progression of disease help to stimulate basic investigations in academia. Without these discoveries, research is hindered and diagnosis and treatment are slowed. In order to better position public–private partnerships to facilitate drug development, it is highly beneficial to take an initial assessment of what aspects of drug discovery and development research academia does best, what aspects the private sector does best, and what aspects are best done truly collaboratively. From there, partners can begin to map out their responsibilities and goals.

Fortunately, voluntary health organizations are helping to bridge the gap in the process of translating basic research into commercially viable product candidates, funding efforts to translate initial scientific discoveries into testable, clinical-stage drug development candidates, biomarkers, and diagnostic tools. This new model of venture philanthropy has been proven on the ground, not only in the pioneering work in polio, but in the current success of organizations like the National Cystic Fibrosis Foundation, whose venture philanthropy efforts have created a broad pipeline of drugs that are advancing rapidly toward the clinic.

Today, an ever-increasing number of voluntary health organizations are developing translational research programs and are looking for new ways to leverage their resources and know-how to fulfill their missions and impact the lives of patients.

FROM PUBLIC TO PRIVATE

The presentations and discussions during the workshop showed that moving into research supported by venture philanthropy involves real risks and unique challenges. The venture philanthropy model calls for voluntary health organizations that are accustomed to working solely with other nonprofits to instead engage directly with the for-profit world, working hand in hand with (and investing in) private industry as they seek to advance drugs into the clinic. The transition from funding nonprofit research to funding for-profit activities is a large one, and it raises

a host of critical legal, accounting, partnership, communication, and related issues. Attendees at the workshop brought with them diverse experiences in setting up venture philanthropy programs and shared their insights, successes, and lessons learned. This workshop summary attempts to gather those experiences and insights into a single source so that they may be called upon by others who are looking to enter this field.

Workshop chair Timothy Coetzee of Fast Forward of the National Multiple Sclerosis Society noted that embracing venture philanthropy does not mean turning away from original and basic science research. He stated in his concluding remarks that scientific discovery is still important, but it is also important to develop new funding models that bring products into the clinic. Voluntary health organizations should "own the strategy" as they embrace venture philanthropy, Coetzee said. They need to set their own agendas and guide the research they fund. It is a message borrowed from modern venture capitalism, where investors aim to deliver not just capital, but "smart capital."

THE DOUBLE BOTTOM LINE

Voluntary health organizations are in a unique position. They work with a double bottom line, looking not just at the dollars and cents but at the real-life impacts on patients. Many organizations receive funding from individuals committed to curing a certain condition and improving lives. That gives them the flexibility to invest in endeavors that might be too risky for private capital. By building upon what is already in place; forming partnerships with academia, industry, and government; and helping set the agenda for research, translational research programs supported by venture philanthropy can play an evermore critical role in advancing new scientific ideas into the clinic, turning them into products and, eventually, improving the health and well-being of patients everywhere.

A

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B

Workshop on Venture Philanthropy Strategies Used by Patient Organizations to Support Translational Research

October 3, 2008
Beckman Center
Irvine, CA

Meeting Goals

- Convene a group of key stakeholders and experts representing a variety of patient organizations involved in venture philanthropy-supported translational research to identify and discuss best practices used that may improve efficiency and effectiveness. Areas that will be discussed include

    - criteria used to determine whether there is sufficient evidence to justify moving into translational research—both translational research from basic to clinical and from clinical research toward practice recommendations for the development of a novel drug and/or biomarker/diagnostic;
    - organizational structure, financial models, legal issues;
    - partnerships, data sharing, and IP; and
    - communication strategies.

Workshop Scenario

Session specific scenarios were developed to help guide remarks and frame the discussions and goals for the workshop. At the heart is a hypothetical patient organization, the Colten Foundation. An individual from this foundation is seeking advice on establishing a translational research program for the development of a novel drug and/or biomarker/diagnostic. The foundation is

trying to plan for and think about the challenges they may encounter and is interested to learn more about how your organization tackled them (where applicable); other best practices, general advice, and lessons learned would also be extremely helpful.

To the best of your knowledge there is no resource currently available that provides advice or "best practices," so it is important that you convey the most critical information that can assist the foundation with its series of decision matrices. The following questions help frame the context:

1. What advice would you want to provide to this individual and his or her organization?
2. What do you wish you had known then that you know now?
3. If I had only known *X*, I could have avoided *Y*.

8:30 Welcome Introductions and Workshop Objectives

TIMOTHY COETZEE, *Chair*
Executive Director
Fast Forward of the National Multiple Sclerosis Society

8:40 Opening Remarks: Opportunities for Foundation Support

JOYCE NELSON
President and Chief Executive Officer
National Multiple Sclerosis Society

9:05 Challenges and Opportunities for Neurological and Mental Health Translational Research: Basic to Clinical Research and Clinical Toward Practice

DENNIS CHOI
Executive Director, Strategic Neurosciences Initiative
Executive Director, Comprehensive Neurosciences Initiative
Emory University

SESSION I: PREPARING FOR A TRANSLATIONAL RESEARCH PROGRAM

Session Objective: Discuss the challenges and issues in the development of a translational research program for the development of either a novel drug or biomarker.

Session I Scenario

An individual from the Colten Foundation, a patient organization that has sufficient resources, has left you a voice mail asking for advice about how they should go about establishing and maintaining a productive and efficient translational research program. They are particularly interested in the criteria considered and the decision matrix you and your organization used to determine whether or not your organization would establish a formal translational research program for the development of a novel drug and/or biomarker/diagnostic.

9:20 Introduction to the Session

MARIA CARRILLO, *Session Chair*
Director, Medical and Scientific Relations
Alzheimer's Association

9:25 Panelist Remarks

RICHARD INSEL
Executive Vice President, Research
Juvenile Diabetes Research Foundation

KATIE HOOD
President and Chief Executive Officer
The Michael J. Fox Foundation for Parkinson's Research

CELIA DOMINGUEZ
Vice President, Chemistry
CHDI Management, Inc.

10:05 Respondent Panel Discussion

LOUIS DEGENNARO
Chief Scientific Officer
Leukemia & Lymphoma Society

CYNTHIA JOYCE
Executive Director
Spinal Muscular Atrophy Foundation

LUCIE BRUIJN
Vice President of Research
Amyotrophic Lateral Sclerosis Association

10:20 Discussion with Attendees

MARIA CARRILLO, *Moderator*
Director, Medical and Scientific Relations
Alzheimer's Association

10:40 BREAK

SESSION II: ORGANIZATIONAL STRUCTURE, LEADERSHIP, AND FINANCIAL MODELS

Session Objective: Explore several elements of how a program should be organized to ensure appropriate expertise and leadership is available. Examine a variety of organizational and financial models that may provide optimal support of translational research programs.

Session II Scenario

The individual from the Colten Foundation who previously spoke to you has used your decision matrix and decided to move ahead with a formal research program. Given how helpful you originally were, they are now asking for more information. In particular they are interested in hearing about models of

organizational and leadership structure and various financial models that you have found to be effective and efficient.

10:55 Introduction to the Session

TIMOTHY COETZEE, *Session Chair*
Executive Director
Fast Forward of the National Multiple Sclerosis Society

11:00 Panelist Remarks

JONATHAN SIMONS
President and Chief Executive Officer
Prostate Cancer Foundation

CYNTHIA JOYCE
Executive Director
Spinal Muscular Atrophy Foundation

11:40 Respondent Panel Discussion

JONATHAN JACOBY
Chief Operating Officer
CollabRx, Inc.

LINDA VAN ELDIK
Professor, Department of Cell and Molecular Biology
Northwestern University Feinberg School of Medicine

11:50 Discussion with Attendees

TIMOTHY COETZEE, *Moderator*
Executive Director
Fast Forward of the National Multiple Sclerosis Society

12:15 LUNCH

SESSION III: LEGAL, ACCOUNTING, AND PROCESS ISSUES

Session Objective: Explore the challenges and opportunities in legal, accounting, and associated process issues that a foundation would need to consider in conducting venture philanthropy translational research programs.

Session III Scenario

You are now very popular with the Colten Foundation! They just sat down with their start-up team and are trying to think about some of the new challenges they may face in terms of appropriate accounting and legal practices, and other process-related issues. They have once again called you to learn more about how you and your organization have navigated these issues.

1:00 Introduction to the Session

TIMOTHY COETZEE, *Session Chair*
Executive Director
Fast Forward of the National Multiple Sclerosis Society

1:05 Panelist Remarks

ANDREA TOBIAS
Portfolio Advisor
Fast Forward, LLC

KENNETH SCHANER
General Counsel
Kenneth Schaner, PC

PETER HEINECKE
Chief Business Officer
Experimed Bioscience, Inc.

1:45 Respondent Panel Discussion

CAROL MIMURA
Assistant Vice Chancellor
Intellectual Property & Industry Research Alliances
University of California, Berkeley

JENNIFER TAYLOR
Associate Director, Program Management
Head of External Alliances
Genomics Institute of the Novartis Research Foundation

1:55 Discussion with Attendees

TIMOTHY COETZEE, *Moderator*
Executive Director
Fast Forward of the National Multiple Sclerosis Society

SESSION IV: PARTNERSHIPS, DATA SHARING, AND IP

Session Objective: Examine a variety of strategies for partnering with nonprofit organizations, the NIH, and industry. Discuss what policies have been effective in facilitating the efficient management of data sharing and IP.

Session IV Scenario

The Colten Foundation has previously been working with just a couple of other researchers or companies, but realizes that in order to develop an effective research program they will need to expand their portfolio of collaborators (e.g., nonprofit organizations, the NIH, therapeutic/diagnostic industry). Of course, this results in more questions than answers, especially around issues of data sharing and IP. They again turn to you to help answer their questions. Are there any common resources available? What are some challenges they may encounter, and how your organization tackled them? How and when should strategic collaborations be established or expanded?

2:10 Introduction to the Session

JUDY ILLES, *Session Chair*
Professor
Neurology
University of British Columbia

2:15 Panelist Remarks

MARGARET ANDERSON
Chief Operating Officer
FasterCures

SHARON HESTERLEE
Vice President, Translational Research
Muscular Dystrophy Association

TRICIA BROOKS
Managing Director, Alliance Development
Biotechnology Industry Organization

2:55 Respondent Panel Discussion

SUSAN WOLF
McKnight Presidential Professor of Law, Medicine, & Public Policy
University of Minnesota School of Law

RUSTY BROMLEY
Chief Operating Officer
Myelin Repair Foundation

3:05 Discussion with Attendees

JUDY ILLES, *Moderator*
Professor
Neurology
University of British Columbia

3:20 BREAK

SESSION V: COMMUNICATIONS

Session Objective: Explore effective communication strategies that can be used to disseminate information on research and other scientific progress to potential and current funders as well as to constituents, while addressing the challenges of funder and constituent expectations.

Session V Scenario

The individual from the Colten Foundation is calling again, and has promised that this will be the last time she will call, but she has one last set of very important questions. Based on everything they have already learned from you, their translational research program is making great headway. They are very excited to share the progress with current and potential funders, as well as their constituents. But, she also recognizes the delicate balance that is needed between demonstrating progress and limiting false expectations. Therefore, she is very interested in discussing effective communication strategies that you have used for disseminating information on research and other progress to potential funders and constituents.

3:30 Introduction to the Session

JONATHAN JACOBY, *Session Chair*
Chief Operating Officer
CollabRx, Inc.

3:35 Panelist Remarks

SHARON TERRY
President and Chief Executive Officer
Genetic Alliance

DAN ZENKA
Vice President, Communications
Prostate Cancer Foundation

CATHY CARLSON
Senior Director, Research Information
Research and Clinical Programs Department
National Multiple Sclerosis Society

4:15 Respondent Panel Discussion

SOPHIA COLAMARINO
Vice President, Research
Autism Speaks

RANDALL CARPENTER
Co-Founder, President, and Chief Executive Officer
Seaside Therapeutics

4:25 Discussion with Attendees

JONATHAN JACOBY, *Moderator*
Chief Operating Officer
CollabRx, Inc.

SESSION VI:
WRAP-UP DISCUSSION

Session Objective: In addition to the areas that have already been discussed, are there additional topics that require exploration? What "next steps" should the IOM's Neuroscience Forum consider as it moves ahead in this area? How can we capture the innovation and ideas discussed during the meeting to assist you and new colleagues working in these areas?

4:40 Discussion with Attendees

TIMOTHY COETZEE, C*hair*
Executive Director
Fast Forward of the National Multiple Sclerosis Society

5:00 ADJOURN

C

Workshop Attendees

Cara Allen
National Institute of Neurological Disorders and Stroke, National Institutes of Health (NIH)

Diane Baker
Genetic Alliance

Karla Blonsky
Biobasix Solutions Inc.

Natasha Bonhomme
Genetic Alliance

Scott Braxton
Excellent Communications, LLC

Greg Dewey
Keck Graduate Institute

Franci Duitch
University of California, Los Angeles

Martin Garcia
Myasthenia Gravis Foundation of California

Les Halberg
Alfred E. Mann Foundation for Biomedical Engineering

Diana Hardy
National Multiple Sclerosis Society

Jessica Harrington
MEP Consulting

Zuzana Hostomska
National Multiple Sclerosis Society

Kerry Howell
Keck Graduate Institute of Applied Life Sciences

Sarah Ingersoll
University of Southern California

Adam Kolom
Cancer Research Institute

James J. Kovach
Buck Institute for Age Research

Dory Kranz
Hydrocephalus Association

Clara Lajonchere
Autism Speaks

Catherine Ley
CollabRx, Inc.

Nan Luke
National Multiple Sclerosis Society

Debra Miller
CureDuchenne

Gary Murray
National Institute of Neurological Disorders and Stroke, NIH

Richard Nakamura
National Institute of Mental Health, NIH

Eric Nicolaides
Wildcat Venture Management

Sandra Noack
CollabRx, Inc.

Jill O'Donnell-Tormey
Cancer Research Institute

William Read
The Flinn Foundation

Peter Saltonstall
National Organization for Rare Disorders

Rae Silver
Columbia University

Edward Spack
SRI International

Kaitlin Thaney
Science Commons

Dan van Kammen
CHDI Foundation, Inc.

D

Biographical Sketches of Invited Speakers, Planning Committee Members, and Staff

INVITED SPEAKERS

Margaret Anderson joined FasterCures in June 2004 as chief operating officer. She comes to the organization after 5 years at the Academy for Educational Development (AED) in Washington, DC. At AED, she was the deputy director and a team leader in the Center on AIDS & Community Health. Between 1995 and 1998, Ms. Anderson was program director for the Society for Women's Health Research. Prior to joining the society, she was a health science analyst at the American Public Health Association from 1992 to 1995, where she managed a programmatic portfolio on HIV/AIDS and other sexually transmitted diseases, infectious diseases, women's health, and public health infrastructure issues. From 1987 to 1991, Ms. Anderson was an analyst and project director at the Congressional Office of Technology Assessment. She currently serves as a member of the Whitman-Walker Clinic institutional review board and has held numerous committee and coalition memberships for federal agencies and professional associations in the biomedical and public health arena. She holds a bachelor's degree from the University of Maryland and a master's degree in science, technology, and public policy from George Washington University's Elliott School of International Affairs.

Russell "Rusty" Bromley is chief operating officer at the Myelin Repair Foundation. Rusty brings a unique combination of business experience in both academic and commercial research environments. His expertise includes the creation and protection of innovative technologies, business development, and marketing strategies for high tech and life sciences

firms. Formerly he was CEO of Lab Velocity, Inc., and Internet information portal for the life sciences research community. Prior to that, he was CEO of Berkshire Holding Corporation, a privately-held, multinational manufacturer of contamination control materials for microelectronics and pharmaceutical production. Bromley's experience also includes 17 years with American Hospital Supply Corporation and Baxter Healthcare, in both the distribution and diagnostics businesses, culminating with 7 years as president of the Burdick and Jackson Division. Bromley holds a degree in biochemistry from Rice University.

Tricia Brooks is the managing director for alliance development at the Biotechnology Industry Organization (BIO). Ms. Brooks brings more than 15 years of public affairs, patient advocacy, and FDA regulatory experience to the position, building coalitions and partnerships with patient advocacy organizations; local, national, and international business organizations; state and federal government relations, and pharmaceutical and biotechnology communities. Most recently, Ms. Brooks was a principal at WHD Government Affairs, developing and implementing public affairs strategies, managing issue campaigns, and representing a variety of clients in Washington while developing public- and private-sector relationships.

Ms. Brooks was part of the team that successfully integrated Michael J. Fox into national campaigns to raise awareness of the embryonic stem cell research debate. Notably, she was the director of government relations for the Christopher and Dana Reeve Foundation (CDRF). Prior to CDRF, Ms. Brooks advocated policies related to biomedical research, including clinical trials and stem cell research, led issue-based campaigns through broad public education initiatives, mobilized grassroots lobbying, and developed diverse coalitions to advocate with both the legislative and executive branches. Ms. Brooks began her career as a regulatory consultant for pharmaceutical development providing strategic, regulatory, and technical assistance to the pharmaceutical and biotech industry. She has a B.A. in political science from Fordham University and an M.A. in public policy from George Washington University.

Lucie Bruijn, Ph.D., joined the Amyotrophic Lateral Sclerosis (ALS) Association in January 2001 as science director and vice president. Prior to joining the association, Dr. Bruijn led a small team at Bristol Myers Squibb developing in vitro and in vivo model systems for neurodegenerative disease. Dr. Bruijn received her bachelor's degree in pharmacy

from Rhodes University, South Africa. She received a master's degree in neuroscience and a Ph.D. in biochemistry, specializing in disease mechanisms of Alzheimer's disease, from the University of London, United Kingdom. At the ALS Association, Dr. Bruijn leads the scientific research enterprise. She has expanded on the existing grant programs, launching a groundbreaking new research initiative, Translational Research to Advance Therapies for ALS (TREAT ALS), with the goal to move treatment options from "bench to bedside."

Cathy Carlson has been communicating science and medicine to nonscientists for over 25 years, largely for nonprofit organizations. She conducted undergraduate lab research in psychobiology and began her career as a science writer in environmental studies before moving into neurology and general medicine. She was a staff science writer and magazine editor for the Muscular Dystrophy Association and a freelance medical writer and journalist before joining the staff of the National Multiple Sclerosis Society's headquarters. Ms. Carlson's unit develops strategies and materials to feed the national website, national magazine, and local chapters. Her unit supports chapters in their research donor development efforts and develops training sessions and materials to a chapter-based volunteer program aimed at building community awareness of the society's research activities and progress.

Randall Carpenter, M.D., is co-founder, president, and CEO of Seaside Therapeutics, a drug discovery company focused on developing novel therapeutics for disorders of brain development such as Fragile X and autism. Dr. Carpenter has over 25 years of experience in medicine, basic science and clinical research, pharmaceutical drug development, and management. He has held a number of leadership positions in the pharmaceutical and biotechnology industries including president and CEO of Sention, vice president of clinical research and development and regulatory affairs at Adolor Corporation, director of clinical research at Astra USA, and member of the Global Therapeutic Area Team at Astra Pain Control Sweden.

Dr. Carpenter has broad experience leading pharmaceutical research and development teams submitting successful INDs, NDAs, and sNDAs. Prior to industry, he held academic faculty appointments at the University of Washington and Wake Forest University. He has coauthored 65 journal articles and several patents and has served as editor-in-chief or on the editorial boards of four medical journals. He has frequently been an

invited speaker at national and international meetings and symposia and has been an invited visiting professor to numerous domestic and international universities and medical schools. Dr. Carpenter is board certified in anesthesiology and pain management and has completed a fellowship in pharmacokinetics at the University of California, San Francisco, and a sabbatical in molecular biology at the University of Washington.

Maria Carrillo, Ph.D., is the director of medical and scientific relations for the Alzheimer's Association national office in Chicago. As such, she has a wide range of responsibilities, including oversight of the association's granting process and communication of scientific findings within and outside the organization. Dr. Carrillo received her Ph.D. from Northwestern University's Institute for Neuroscience in 1996. Since graduating from Northwestern, she completed a postdoctoral fellowship in the Neurology Department at Rush-Presbyterian-St. Luke's Medical Center in Chicago, where she was later hired as an assistant professor in the Department of Neurological Sciences. During this time she published a dozen papers and book chapters on aspects of memory and was coinvestigator on two grants to study memory mechanisms in Parkinson's disease and anatomical and physiological correlates of cognitive function in Alzheimer's. As the director of medical and scientific relations, she is responsible for overseeing the Scientific Grant Program, the mechanism through which the association funds research applications. In addition to ensuring the smooth review of applications and distribution of awards to successful applicants, she is responsible for sharing results and ongoing investigations with a wide range of constituents.

Dennis W. Choi, M.D., Ph.D., graduated from Harvard College in 1974 and received the M.D. and Ph.D. degrees in 1978 (the latter in pharmacology) from Harvard University and the Harvard–MIT Program in Health Sciences and Technology. After completing residency and fellowship training in neurology at Harvard, he joined the faculty at Stanford University and began research into the mechanisms underlying pathological neuronal death. In 1991, he joined Washington University Medical School as head of the Neurology Department; there he also established the Center for the Study of Nervous System Injury and directed the McDonnell Center for Cellular and Molecular Neurobiology. From 2001 until 2006, he was executive vice president for neuroscience at Merck Research Labs. Dr. Choi is currently vice president for academic

health affairs, Woodruff Health Sciences Center, and executive director, Comprehensive Neurosciences Initiative, at Emory University.

He is a fellow of the American Association for the Advancement of Science and a member of the Institute of Medicine, the Executive Committee of the Dana Alliance for Brain Initiatives, and the College of Physicians of Philadelphia. He has served as president of the Society for Neuroscience, vice-president of the American Neurological Association, and chairman of the U.S./Canada Regional Committee of the International Brain Research Organization. He has also served on the National Academy of Sciences' Board on Life Sciences and councils for the National Institute of Neurological Disorders and Stroke, the Society for Neuroscience, the Winter Conference for Brain Research, the International Society for Cerebral Blood Flow and Metabolism, and the Neurotrauma Society. He has been a member of advisory boards for the Christopher Reeve Paralysis Foundation, the Grass Foundation, the Hereditary Disease Foundation, the Spinal Muscular Atrophy Foundation, the Harvard–MIT Program in Health Sciences and Technology, the Queen's Neuroscience Institute in Honolulu, the Max Planck Institute in Heidelberg, Germany, the Korea Institute for Advanced Study (KIAS) in Seoul, and the U.S. Food and Drug Administration, as well as for several university-based research consortia, biotechnology companies, and pharmaceutical companies.

Timothy Coetzee, Ph.D., is the executive director of Fast Forward, LLC, a venture philanthropy of the National Multiple Sclerosis (MS) Society. In this capacity, Dr. Coetzee is responsible for the society's strategic funding of biotechnology and pharmaceutical companies as well as partnerships with the financial and business communities. Prior to assuming his current position, Dr. Coetzee led the society's translational research initiatives on nervous system repair and protection in Multiple Sclerosis (MS) as well as the society's programs to recruit and train physicians and scientists in MS research.

Dr. Coetzee received his Ph.D. in molecular biology from Albany Medical College in 1993 and has since been involved in the field of MS research. He was a research fellow in the laboratory of society grantee Dr. Brian Popko at the University of North Carolina at Chapel Hill, where he received an Advanced Postdoctoral Fellowship Award from the society. After completing his training with Dr. Popko, Dr. Coetzee joined the faculty of the Department of Neuroscience at the University of Connecticut School of Medicine, where he conducted research that applied

new technologies to understand how myelin is formed in the nervous system. He is the author of a number of research publications on the structure and function of myelin. Dr. Coetzee joined the National MS Society's home office staff in the fall of 2000.

Sophia Colamarino, Ph.D., graduated with dual degrees in biological sciences and psychology from Stanford University. Following her undergraduate degrees she received her Ph.D. in neurosciences from the University of California, San Francisco (UCSF), working with distinguished neuroscientist Marc Tessier-Lavigne, Ph.D. Her thesis focused on neurodevelopment, specifically the development of brain connectivity. After receiving her Ph.D., Dr. Colamarino conducted research at the Telethon Institute for Genetics and Medicine in Milan, Italy, led by human geneticist Andrea Ballabio, M.D. She then returned to the United States to begin her fellowship at the Salk Institute in La Jolla, California, where she studied adult neural stem cells in the laboratory of stem cell pioneer Fred H. Gage, Ph.D. Sophia has published in such journals as *Cell, Nature,* and *Nature Medicine.* After 16 years of laboratory research, she joined the Cure Autism Now Foundation (CAN) in November 2004 as science director. During her tenure at CAN, she oversaw a large growth in the science program and developed several important initiatives including the Neuropathology Workgroup, a collaborative effort to understand the cellular and molecular basis of brain enlargement, the first Environmental Innovator Award, and research summit meetings on immunology and neuroimaging, among others. Currently, as vice president of research for Autism Speaks, the largest private funder of autism biomedical research worldwide, Dr. Colamarino's many responsibilities include management and oversight of the Autism Speaks' biology portfolio and the new High Risk/High Impact program. She has also become well known for her ability to communicate science to lay audiences. She grew up in San Francisco and currently resides in Los Angeles.

Louis DeGennaro, Ph.D., is currently responsible for the administration of the Leukemia & Lymphoma Society's (LLS's) research grant programs that support research leading into the prevention, diagnosis, and cure of leukemia, lymphoma, and myeloma. He also directs LLS's Therapy Acceleration Program*,* which supports private-sector and academic-based projects with the goal of moving more blood cancer therapies into the development pipeline. Dr. DeGennaro has more than 20 years of research and drug development experience in academic and private-sector

settings. He received his Ph.D. in biochemistry from the University of California, San Francisco, and did his postdoctoral research at Yale University School of Medicine. His previous academic positions include research group leader, Max Planck Institute in Munich, Germany, and associate professor of neurology and cell biology, University of Massachusetts Medical School. Dr. DeGennaro's private-sector positions include senior director of molecular genetics at Wyeth Pharmaceuticals, Princeton, New Jersey, and executive vice president for research and development, SynX Pharma, Inc., in Toronto, Canada.

Celia Dominguez, Ph.D., is currently vice president of chemistry at CHDI Management, Inc., a privately held, not-for-profit organization that is pursuing a biotech approach to the discovery and development of drugs that prevent or slow the progression of Huntington Disease. Dr. Dominguez has 17 years of drug discovery and development experience in the pharmaceutical biotechnology sector with Amgen and DuPont Merck, where she held positions of increasing responsibility. At DuPont Merck, she was part of the team that discovered a potent and selective FXa inhibitor, which eventually led to the identification of a clinical candidate currently in phase 3 clinical trials. Dr. Dominguez received a B.S. in chemistry from Rutgers University, a Ph.D. in synthetic organic chemistry from Brown University, and postdoctoral training at National Institutes of Health/National Institute of Diabetes and Digestive and Kidney Diseases. She is a member of the American Chemical Society, the American Association for the Advancement of Science, the Society for Neuroscience, and the Expert Opinion on Therapeutic Patents editorial board for patent selections.

Peter Heinecke, J.D., is an independent business and legal adviser to early-stage life science and high-tech companies and currently serves as chief business officer of Experimed Bioscience, Inc., an early-stage therapeutics company. Prior to Experimed, Mr. Heinecke was a founder and vice president of finance and corporate development at Aviir, Inc., a venture-backed molecular diagnostics company. He was a corporate and licensing attorney in private practice for over 10 years at the law firms of Wilson Sonsini Goodrich and Rosati, PC, and Kirkpatrick & Lockhart Nicholson Graham, LLP. In his practice he represented a wide variety of public and private companies on matters such as venture capital financings, technology licensing, mergers and acquisitions, SEC compliance, and corporate formation and governance. Mr. Heinecke also worked for

2 years as an investment banker with Morgan Stanley & Co. He holds a J.D. and an M.B.A. from the University of Chicago and an A.B. in politics from Princeton University.

Sharon Hesterlee, Ph.D., is vice president of translational research at the Muscular Dystrophy Association. Dr. Hesterlee received her Ph.D. in neuroscience in 1999 from the University of Arizona, where she studied neural development and received funding from a Flinn Foundation Training Grant. From 2000 to 2006, she served as the Muscular Dystrophy Association's director of research development. In that position she developed and oversaw an $8 million translational research program aimed at increasing industry participation in drug development for rare disease. She has been involved in the planning of several meetings to identify and remove barriers to therapy development for neuromuscular disease, and she serves on numerous advisory boards including the Department of Health and Human Services federal advisory committee for muscular dystrophy. In 2006, Dr. Hesterlee was appointed vice president of translational research and, in addition to overseeing that program, is currently directing major collaborations in the areas of Duchenne muscular dystrophy, Friedreich's ataxia, spinal muscular atrophy, and amyotrophic lateral sclerosis (ALS).

Katie Hood is chief executive officer of The Michael J. Fox Foundation for Parkinson's Research (MJFF). She has played critical roles in shaping MJFF's strategy of aggressively intervening to close critical gaps that slow potential treatments on their path from the laboratory to Parkinson's patients, as well as in building a team of in-house research experts needed to implement that strategy. Prior to joining the foundation in September 2002, Ms. Hood was employed as a consultant at Bain & Company in New York City, doing work in the consumer products, financial services, and nonprofit sectors. She has also served as an analyst in the credit department of Goldman, Sachs & Co., and as a program coordinator with Duke University's Hart Leadership Program. In August 2008, Ms. Hood was named to the Advisory Council to the National Institute of Neurological Disorders and Stroke (NINDS), an 18-member board that advises the secretary of health and human services, the director of the National Institutes of Health, and the director of NINDS on research funding prioritization and related matters for neurological diseases, including Parkinson's disease. She also is a member of the board of directors of the Parkinson's Action Network (PAN). She graduated from the

Harvard Business School and holds a B.A. in public policy studies from Duke University in Durham, North Carolina.

Judy Illes, Ph.D., is professor of neurology and Canada Research Chair in Neuroethics for the National Core for Neuroethics at the University of British Columbia. Dr. Illes received her doctorate in hearing and speech sciences from Stanford University in 1987, with a specialization in experimental neuropsychology. She returned to Stanford University in 1991 to help build the research enterprise in imaging sciences in the Department of Radiology. She also cofounded the Stanford Brain Research Center (now the Neuroscience Institute at Stanford) and served as its first executive director between 1998 and 2001. Most recently, she was acting associate professor of pediatrics (medical genetics) and director of the program in neuroethics at the Stanford Center for Biomedical Ethics.

Dr. Illes has written numerous books, edited volumes, and articles. She is the author of *The Strategic Grant Seeker: Conceptualizing Fundable Research in the Brain and Behavioral Sciences*; special guest editor of *Topics of Magnetic Resonance Imaging*, "Emerging Ethical Challenges in MR Imaging"; and *Brain and Cognition*, "Ethical Challenges in Advanced Neuroimaging." Her latest book, *Neuroethics: Defining the Issues in Theory, Practice and Policy*, was published by Oxford University Press in January 2006. Dr. Illes is cochair of the Committee on Women in Neuroscience of the Society for Neuroscience, a member of the Internal Advisory Board of the Institute of Neurosciences, Mental Health and Addiction of the Canadian Institutes of Health Research, and a member of the Dana Alliance for Brain Initiatives.

Richard Insel, M.D., is the executive vice president of research at the Juvenile Diabetes Research Foundation (JDRF), where he has responsibility for heading up the strategic direction and oversight of JDRF research projects. Prior to joining the JDRF, Dr. Insel was director of the University of Rochester Medical Center's Center for Human Genetics and Molecular Pediatric Disease. He joined JDRF after 26 years at the University of Rochester Medical Center, where he was a member of that institution's departments of Pediatrics and Microbiology and Immunology. He has been founding director of the Center for Human Genetics and Molecular Pediatric Disease since 2000. Dr. Insel also serves on the National Advisory Allergy and Infectious Diseases Council of the National Institutes of Health. In addition to his university research experience, Dr. Insel was scientific cofounder of Praxis Biologics, a

biotechnology company established in 1983 and subsequently acquired by Wyeth, the global pharmaceutical and health care products company. He has also served as a visiting associate professor of biochemistry and biophysics at the College of Physicians and Surgeons of Columbia University, a fellow in Pediatrics (Research) at Harvard Medical School, a fellow in Medicine (Immunology) at Children's Hospital Medical Center in Boston, and in the Laboratory of Parasitic Immunochemistry at the Centers for Disease Control and Prevention in Atlanta.

Jonathan Jacoby is the chief operating officer of CollabRx, Inc. He has worked as an organizational entrepreneur, executive, and strategic planning consultant for nonprofit organizations. Mr. Jacoby was cofounder and former CEO of the Hide & Seek Foundation for Lysosomal Disease Research, a nonprofit foundation created by parents, scientists, business leaders, and philanthropists. As the chief staff person of the foundation, he worked to accelerate medical research and scientific innovation. He also founded and served as executive director of the Israel Policy Forum, a bipartisan advocacy group and think tank, and the New Israel Fund, a philanthropic foundation. He also served as a partner at the consulting firm Bronznick Jacoby, LLC, working with not-for-profit organizations to launch new ventures and programs, position organizations for growth, and guide agencies through periods of transition. Mr. Jacoby holds an M.E. from Harvard University, a B.A. from UCLA, and a B.Lit. from the University of Judaism.

Cynthia Joyce is the executive director of the Spinal Muscular Atrophy (SMA) Foundation and joined as the first employee of the organization, where she has focused on building momentum in research and drug development for SMA. Ms. Joyce was previously the director of the American Academy of Neurology (AAN) Foundation, which supports public education and research in all areas of neurology. Prior to working at the AAN, Ms. Joyce served as a product director for Cephalon, a leading biopharmaceutical company in the Philadelphia biotech corridor, and at Ciba Pharmaceuticals (now Novartis) in New Jersey. She holds a B.S. from the University of Chicago and an M.S. in botany from the University of Minnesota. She has served as an adviser to numerous organizations including the National Institute of Neurological Disorders and Stroke, the Epilepsy Foundation, the Amyotrophic Lateral Sclerosis Association, and many others. She is currently serving on the board of directors of the American Society for Experimental Neurotherapeutics.

Carol Mimura, Ph.D., is the assistant vice chancellor for Intellectual Property & Industry Research Alliances (IPIRA) at the University of California, Berkeley (UC Berkeley). IPIRA is the portal for industry access to Berkeley's preeminent faculty and research capabilities. Dr. Mimura has a B.S. Yale University in molecular biophysics and biochemistry and a Ph.D. in biology (biochemistry and microbiology concentration) from Boston University. She was an NIH-sponsored postdoctoral fellow and research scientist at UC Berkeley in biochemistry and in chemical biodynamics. She served on the board of directors of the Children's Hospital Research Institute in Oakland, California, and as a board member (the chancellor's alternate) of BayBio, the regional voice of biotechnology in northern California. She is a former executive director of UC Berkeley's Office of Technology Licensing. Prior to her positions at UC Berkeley, Dr. Mimura was an analyst at Technology Forecasters, a consultant to Cor Therapeutics and Genomyx, and a writer for the *Genetic Engineering News*.

Joyce Nelson is president and chief executive officer of the National Multiple Sclerosis Society. Ms. Nelson started her 21-year career with the society as development manager with the northern California chapter. After 2 years in that position, she was selected to be executive director of the mid-America chapter, serving the greater Kansas City area, western Missouri, and eastern Kansas. In 1991, she joined the society's national staff and relocated to Denver, Colorado, where she established the position of national director of campaign development. Three years later she was promoted to vice president of chapter programs. For the past 5 years, she has been vice president of field operations, encompassing all responsibilities for the society's fund-raising and for its relations with chapters. During this time she was instrumental in managing significant organizational initiatives including the Research Challenge of Champions, the Promise: 2010 research campaign, the Corporate Star Program to recognize outstanding corporate commitment to the Multiple Sclerosis cause, and the determination of society governance practices.

Kenneth Schaner, J.D., formed his own firm to specialize in the representation of tax-exempt entities at the beginning of 2008, after over 30 years as a partner at Swidler Berlin and Bingham McCutchen. In almost 40 years of private practice, Mr. Schaner has represented many for-profit and nonprofit entities in the corporate and tax aspects of a wide variety of agreements, transactions, financings, licenses, mergers, and acquisi-

tions. Since 1983, he has served as general counsel to the Cystic Fibrosis Foundation (CFF). In that capacity, he represented CFF in its first venture philanthropy transaction, with Aurora Biosciences Corporation. Since then, he has represented CFF, the Juvenile Diabetes Research Foundation, the Stanley Medical Research Institute, the National Neurovision Research Institute, the National Multiple Sclerosis Society, and others in numerous venture philanthropy transactions and related legal matters.

Mr. Schaner began his career at the Internal Revenue Service's legislative and regulations division. During his time with the IRS, he worked on the 1969 Tax Reform Act and was one of the principal drafters of the then-new private foundation provisions. In 1982, he and several others founded Swidler Berlin. As a partner in that firm, he served at various times as its managing member and chairman of its corporate group. After Swidler Berlin's merger with Bingham McCutchen in 2006, Mr. Schaner became a partner at Bingham until he formed his new firm in 2008 to focus his representation on tax-exempt organizations while applying a rate structure that would be uneconomic for the larger firms.

Jonathan Simons, M.D., is chief executive officer and president of the Prostate Cancer Foundation (PCF). Dr. Simons was distinguished professor of hematology and oncology at the Emory University School of Medicine and professor of biomedical engineering and materials sciences at the Georgia Institute of Technology. He is the founding director of the Winship Cancer Institute at Emory University in Atlanta and codirector of the National Cancer Institute Center for Cancer Nanotechnology Excellence at Emory and Georgia Tech. Dr. Simons has been affiliated with the PCF since 1995 when, as a young assistant professor, he received his first research award from the PCF Competitive Awards Program for his research in genetic therapy for advanced prostate cancer. He received a competitive award again in 1996, 1997, and 1998 and was coleader of the Johns Hopkins site in the PCF Therapy Consortium. He has served as a reviewer for both the 2005 Competitive Awards Program and the 2006 Donald S. Coffey Career Development Program. Dr. Simons himself was also a recipient of the PCF Donald S. Coffey Award for Physician-Scientists.

Jennifer Taylor, Ph.D., is the head of external alliances and associate director of program management at the Genomics Institute of the Novartis Research Foundation (GNF) in San Diego, California. GNF's mission

is to apply state-of-the-art technologies in chemistry, biology, automation, and information sciences to explore complex biomedical problems in cancer biology, immunology, neuroscience, and metabolic as well as infectious diseases. These discoveries are being translated into human therapeutics through an internal preclinical drug discovery effort coupled with further development activities in collaboration with Novartis Pharmaceuticals. In addition to collaborations within Novartis, GNF also fosters active partnerships with researchers at academic institutions, nonprofit foundations, and federal granting agencies. In her role at GNF, Dr. Taylor is responsible for managing preclinical drug discovery programs with Novartis Pharmaceuticals, nonprofit foundations, and government agencies. Before joining GNF in 2004, she was a senior research scientist at Vertex Pharmaceuticals in San Diego. She received her B.A. in biology from Wellesley College and her Ph.D. in cellular and molecular biology from the University of Pennsylvania.

Sharon Terry is president and CEO of the Genetic Alliance, a network transforming health by promoting an environment of openness centered on the health of individuals, families, and communities. She is the founding executive director of PXE International, a research advocacy organization for the genetic condition pseudoxanthoma elasticum (PXE). Following the diagnosis of their two children with PXE in 1994, Ms. Terry, a former college chaplain, and her husband, founded and built a dynamic organization that fosters ethical research and policies and provides support and information to members and the public. Ms. Terry is at the forefront of consumer participation in genetics research, services, and policy and serves as a member of many of the major governmental advisory committees on medical research, including liaison to the Secretary's Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children and the National Advisory Council for Human Genome Research, National Human Genome Research Institute at the National Institutes of Health.

Ms. Terry is a cofounder of the Genetic Alliance BioBank, a centralized biological and data (consent/clinical/environmental) repository catalyzing translational genomic research on rare genetic diseases. The BioBank works in partnership with academic and industrial collaborators to develop novel diagnostics and therapeutics to better understand and treat these diseases. Along with the other coinventors of the gene associated with PXE (ABCC6), Ms. Terry holds the patent for the invention.

She codirects a 33-lab research consortium and manages 52 offices worldwide for PXE International.

Andrea Tobias, Ph.D., is currently the portfolio adviser to Fast Forward, LLC, the philanthropic venture arm of the U.S. National Multiple Sclerosis Society. She is also a part-time venture partner with Brandon Capital Partners, a life sciences fund in Sydney, Australia. She previously was a partner at CMEA Ventures (San Francisco) from 2001 to 2006. Prior to this, Dr. Tobias spent 4 years in venture capital as an assistant director for Apax Partners & Cie Ventures (Paris) and with Abingworth Management (London). She also held management positions for 10 years in the U.S. biotech industry. She was director of strategic development for Chiron Corporation and manager of new research identification for Genentech. In addition, she served as the research and development liaison for foreign subsidiaries in Canada, Japan, and Switzerland. Dr. Tobias has a degree in physiology/anatomy from the University of California, Berkeley, and a Ph.D. in endocrinology from the University of California, San Francisco, School of Medicine.

Linda Van Eldik, Ph.D., is codirector of the Center for Drug Discovery and Chemical Biology at Northwestern University, associate director of the Cognitive Neurology and Alzheimer's Disease Center, and professor of cell and molecular biology at Northwestern University Feinberg School of Medicine in Chicago. Dr. Van Eldik has published peer-reviewed articles in neuroscience, glia cell biology, signal transduction, virology, and drug discovery. Dr. Van Eldik received her Ph.D. in microbiology/immunology from Duke University in 1977, followed by postdoctoral training at the Rockefeller University from 1978 to 1981, where she was awarded a National Science Foundation postdoctoral fellowship and National Research Service Award in cell biology from the National Institutes of Health. She later held the positions of assistant professor, associate professor, and professor of pharmacology and cell biology at Vanderbilt University School of Medicine and was an associate investigator with the Howard Hughes Medical Institute before moving to Northwestern University Feinberg School of Medicine in Chicago in 1994.

Susan Wolf, J.D., joined the University of Minnesota faculty in 1993 and is the McKnight Presidential Professor of Law, Medicine, & Public Policy and the Faegre & Benson Professor of Law. She is the founding

director of the Joint Degree Program in Law, Health, & the Life Sciences and the founding chair of the Consortium on Law and Values in Health, Environment, & the Life Sciences. She is also a professor of medicine in the University's medical school and a faculty member in the University's Center for Bioethics. Professor Wolf received an A.B. degree, summa cum laude, from Princeton University and a J.D. degree from Yale Law School. She clerked for Judge Leonard B. Sand of the U.S. District Court for the Southern District of New York and then practiced with the New York law firm of Paul, Weiss, Rifkind, Wharton & Garrison from 1981 to 1984. In 1984–1985, Professor Wolf was a National Endowment for the Humanities Fellow at the Hastings Center in New York, a senior bioethics research institute. She then became the center's associate for law. She also taught law and medicine at New York University School of Law as an adjunct associate professor from 1987 to 1992. In 1992–1993, Professor Wolf was a fellow at Harvard University in the Program in Ethics and the Professions. She currently serves as a member of the Law & Neuroscience Project funded by the MacArthur Foundation and as a senior consultant to the Hastings Center on its project on guidelines for end-of-life care, funded by the Donaghue Foundation and Sussman Trust.

Dan Zenka is vice president of communications at the Prostate Cancer Foundation (PCF). Mr. Zenka brings more than 25 years of international public relations, brand development, and communications strategy experience to his role at PCF. Prior to joining PCF, he was director of public relations and brand management at FEI, an Oregon-based technology company that develops high-end equipment for enabling nanoscale exploration, discovery, and development in the areas of life science, electronics, and general industry. While at FEI he also headed the communications and brand task force for the Oregon Nanoscience and Microtechnologies Institute (ONAMI), a unique, publicly funded consortium of leading academic research institutions, corporations, and the Pacific Northwest National Laboratory, chartered to accelerate nanotechnology development in the Northwest. Mr. Zenka has also held management positions at Bioject Medical Systems and Lattice Semiconductor Corporation and consulted to Philips Medical Systems in the Netherlands. He is a graduate of the University of Southern California with degrees in journalism/public relations and speech communications. He is also an accredited member of the Public Relations Society of America. His articles on technology and communications have been published by *PR Tactics*, *Nanotech Briefs*, and *Nanotechnology Now*.

PLANNING COMMITTEE MEMBERS

Timothy Coetzee, Ph.D. (*Co-chair*), biography in Invited Speakers section.

William H. Thies, Ph.D. (*Co-chair*), is vice president for medical and scientific relations at the Alzheimer's Association, where he oversees the world's largest private, nonprofit Alzheimer's disease research grants program. Under his direction, the organization's annual grant budget has doubled, and the program has designated special focus areas targeting the relationship between cardiovascular risk factors and Alzheimer's disease, caregiving and care systems, and research involving diverse populations. He played a key role in launching *Alzheimer's & Dementia: The Journal of the Alzheimer's Association* and in establishing the Research Roundtable, a consortium of senior scientists from industry, academia, and government who convene regularly to explore common barriers to drug discovery. In previous work at the American Heart Association (AHA) from 1988 to 1998, Dr. Thies formed a new stroke division that recently became the American Stroke Association. He also built the Emergency Cardiac Care Program, a continuing medical education program that trains over 3 million professionals annually. He has worked with the National Institute of Neurological Disorders and Stroke to form the Brain Attack Coalition. Prior to joining the AHA, he held faculty positions at Indiana University in Bloomington and the University of Pittsburgh. Dr. Thies earned a B.A. in biology from Lake Forest College, Lake Forest, Illinois, and a Ph.D. in pharmacology from the University of Pittsburgh School of Medicine.

Huda Akil, Ph.D., is the Gardner Quarton Distinguished University Professor of Neuroscience and Psychiatry at the University of Michigan and the codirector of the Molecular and Behavioral Neuroscience Institute. Dr. Akil has made seminal contributions to the understanding of the neurobiology of emotions, including pain, anxiety, depression, and substance abuse. Early on, she focused on the role of the endorphins and their receptors in pain and stress responsiveness. Dr. Akil's scientific contributions have been recognized with numerous honors and awards. These include the Pacesetter Award from the National Institute on Drug Abuse in 1993 and, with Dr. Stanley Watson, the Pasarow Award for Neuroscience Research in 1994.

In 1998, Dr. Akil received the Sachar Award from Columbia University and the Bristol Myers Squibb Unrestricted Research Funds Award. Dr. Akil is past president of the American College of Neuropsychopharmacology (1998) and past president of the Society for Neuroscience (2004), the largest neuroscience organization in the world with over 35,000 members. She was elected as a fellow of the American Association for the Advancement of Science in 2000. In 1994, she was elected to the membership of the Institute of Medicine (IOM) of the National Academy of Sciences and is currently a member of its council. More recently (2004), she was elected to the American Academy of Arts and Sciences.

Daniel J. Burch, M.D., is executive vice president of research and development and chief medical officer of CeNeRx Biopharma. Dr. Burch holds an M.D. from Vanderbilt University and an M.B.A. from the Wharton School, University of Pennsylvania. He completed a residency in internal medicine at Vanderbilt University School of Medicine and a fellowship in infectious diseases at Washington University School of Medicine. Dr. Burch has worked in the pharmaceutical industry for a total of 15 years at Abbott Laboratories, SmithKlineBeecham, and GlaxoSmithKline (GSK). His most recent post at GSK was senior vice president, Neurosciences Medicines Development Centre. He was appointed to his current position in 2007.

Dennis W. Choi, M.D., Ph.D., biography in Invited Speakers section.

Judy Illes, Ph.D., biography in Invited Speakers section.

Walter Koroshetz, M.D., is the deputy director of the National Institute of Neurological Disorders and Stroke of the National Institutes of Health. He is an internationally renowned neurologist and outstanding investigator and administrator. Prior to his appointment, Dr. Koroshetz was vice chair of the neurology service and director of stroke and neurointensive care services at Massachusetts General Hospital (MGH). He was also a professor of neurology at Harvard Medical School and has led neurology resident training at MGH since 1990.

Dr. Koroshetz graduated from Georgetown University and received his medical degree from the University of Chicago. He trained in internal medicine at the University of Chicago and MGH. He trained in neurology at MGH, after which he did postdoctoral studies in cellular neuro-

physiology at MGH and the Neurobiology Department at Harvard. He joined the neurology staff, first in the Huntington's Disease unit and then in the stroke and neurointensive care service. During his career Dr. Koroshetz has conducted basic electrophysiology research in cell membranes and in cultures of nerve cells and glial cells (which support nerve cells). His clinical research has focused on finding new treatments for patients with Huntington's Disease and stroke.

Story C. Landis, Ph.D., has been director of the National Institute of Neurological Disorders and Stroke (NINDS) since September 1, 2003. As the director of the NINDS, Dr. Landis oversees an annual budget of $1.5 billion and a staff of more than 900 scientists, physician-scientists, and administrators. The institute supports research by investigators in public and private institutions across the country, as well as by scientists working in its intramural laboratories and branches in Bethesda, Maryland. Since 1950, the institute has been at the forefront of U.S. efforts in brain research.

Dr. Landis joined the NINDS in 1995 as scientific director and worked with former institute director Zach W. Hall, Ph.D., to coordinate and reengineer the institute's intramural research programs. Between 1999 and 2000, under the leadership of NINDS director Gerald D. Fischbach, M.D., she led the movement, together with the National Institute of Mental Health scientific director Robert Desimone, Ph.D., to bring some sense of unity and common purpose to 200 laboratories from 11 different NIH institutes, all of which conduct leading-edge clinical and basic neuroscience research.

A native of New England, Dr. Landis received her undergraduate degree in biology from Wellesley College in 1967 and her master's degree (1970) and Ph.D. (1973) from Harvard University, where she conducted research on cerebellar development in mice. After postdoctoral work at Harvard University studying transmitter plasticity in sympathetic neurons, she served on the faculty of the Harvard Medical School Department of Neurobiology.

In 1985, she joined the faculty of Case Western Reserve University School of Medicine in Cleveland, Ohio, where she held many academic positions including associate professor of pharmacology; professor and director of the Center on Neurosciences; and chairman of the Department of Neurosciences, a department she was instrumental in establishing. Under her leadership, Case Western's neuroscience department achieved worldwide acclaim and a reputation for excellence.

Throughout her research career, Dr. Landis has made many fundamental contributions to the understanding of developmental interactions required for synapse formation. She has garnered many honors and awards and is an elected fellow of the Academy of Arts and Sciences, the American Association for the Advancement of Science, and the American Neurological Association. In 2002, she was named the president-elect of the Society for Neuroscience.

Richard Nakamura, Ph.D., is the deputy director of the National Institute of Mental Health (NIMH). He has served in the position of deputy director of NIMH since 1997 and NIMH acting director from 2001 to 2002. He has played a key role in revitalizing both NIMH's extramural and intramural research programs. In addition, he has been at the forefront of efforts to speed the translation of scientific knowledge into clinical practice and to transmit these advances to Congress and the public.

Arriving at NIMH in 1976 as a postdoctoral fellow in the intramural Laboratory of Neuropsychology, Dr. Nakamura conducted behavioral and physiological studies in non-human primates to understand cognitive processing in the brain. He moved to NIMH headquarters in 1986, serving as chief of the Behavioral and Integrative Neuroscience Research Branch in the early 1990s and later as associate director of science policy and program planning.

Rae Silver, Ph.D., is Helene L. and Mark N. Kaplan Professor of Natural and Physical Sciences and holds joint appointments at Barnard College and Columbia University. Dr. Silver is a fellow of the American Academy of Arts and Sciences, American Association of Arts and Sciences. She has participated extensively in scientific and educational activities including serving as chair for NASA's Research Maximization and Prioritization Committee reviewing scientific priorities for the *International Space Station,* as well as chair, External Advisory Committee, National Science Foundation Center for the Study of Biological Rhythms at the University of Virginia. Dr. Silver has been a search committee member for editors of journals, a Society for Neuroscience program committee member (Theme E—Autonomic and Limbic System), department chairs and provost at various institutions, and panel member of a number of committees, including NASA: International Space Station Cost and Management Evaluation Task Force, member Georgia State, Emory and other colleges, National Science Foundation Center for Be-

havioral Neuroscience External Advisory Board Society for Neuroscience Education Committee, and Ford Foundation Minority Fellowship review panel. She was also president of Society Research in Biological Rhythms. As senior adviser at the National Science Foundation (NSF), she worked with NSF staffers in all the scientific directorates to create a series of workshops to examine opportunities for the next decade in making advances in neuroscience through the joint efforts of biologists, chemists, educators, mathematicians, physicists, psychologists, and statisticians.

Dr. Silver's studies of the biological clock in the suprachiasmatic nucleus of the brain were the first to conclusively demonstrate that this brain tissue can be readily transplanted and restore function at a very high success rate in an animal model. The laboratory is renowned for analysis of the input, output, and intraneuronal circuits underlying the function of the brain's master clock. A second line of research entails the study of mast cells (renowned for their role in producing allergic reactions) in modulating brain function and as a major source of brain histamine. The research has been supported without interruption by NIH and NSF, among other sources.

Dr. Silver is deeply committed to educating undergraduate and graduate students, both at the national and institutional level and in the hands-on context of the laboratory. Consistent with this interest, she created the undergraduate program in quantitative reasoning at Barnard College and published, with colleagues, studies of mathematical learning. She initiated the undergraduate major in neuroscience, serving as its first program director. She also served as director of the graduate program in psychology at Columbia University.

Christian G. Zimmerman, M.D., FACS, M.B.A., is chairman and founder of the Idaho Neurological Institute (INI), adjunct professor of psychology at Boise State University, and past chief executive officer of Neuroscience Associates. He has also served as a board member for the Idaho State Board of Health and Welfare. Dr. Zimmerman established the INI research facility to focus on nervous system injury, repair, and neuroplasticity. He leads its various interdisciplinary research teams and is coprofessor for biology and cognitive neuroscience research at the facility. Research projects include a 20-year longitudinal study of traumatic brain injury and investigations of spinal injury, stroke, aneurysms, arterial thrombolytic therapy intervention, neuropathology, central nervous system tumors, sleep disorders, deep brain stimulation, movement disor-

ders, and five TATRC telemedicine grants. In his role as INI chairman, he has facilitated numerous symposia and workshops to provide educational opportunities for medical professionals and for the general public. Additionally, he chairs prevention programs for Idaho's youth such as Think First. Dr. Zimmerman is a diplomate of the American Board of Neurological Surgery and Pain Management and a fellow of the American College of Surgeons and Physician Executives. He received his master's of business administration from Auburn University.

STUDY STAFF

Bruce M. Altevogt, Ph.D., is a senior program officer on the Board on Health Sciences Policy at the IOM. His primary interests focus on policy issues related to basic research and preparedness for catastrophic events. He received his doctorate from Harvard University's program in neuroscience. Following more than 10 years of research, Dr. Altevogt joined the National Academies as a science and technology policy fellow with the Christine Mirzayan Science & Technology Policy Graduate Fellowship Program. Since joining the Board on Health Sciences Policy, he has been a program officer on multiple IOM studies, including Sleep Disorders and Sleep Deprivation: An Unmet Public Health Problem, the National Academies' Guidelines for Human Embryonic Stem Cell Research: 2007 Amendments, and Research Priorities in Emergency Preparedness and Response for Public Health Systems. He is currently serving as director of the Forum on Medical and Public Health Preparedness for Catastrophic Events and the Neuroscience and Nervous System Disorders Forum and as a costudy director on the National Academy of Sciences Human Embryonic Stem Cells Research Advisory Committee. He received his B.A. from the University of Virginia in Charlottesville, where he majored in biology and minored in South Asian studies.

Andrew M. Pope, Ph.D., is the director of the Board on Health Sciences Policy at the IOM. With a Ph.D. in physiology and biochemistry, his primary interests are in science policy, biomedical ethics, and the environmental and occupational influences on human health. During his tenure at the National Academies and since 1989 at the IOM, Dr. Pope has directed numerous studies on topics that range from injury control, disability prevention, and biologic markers to the protection of human subjects of research, NIH priority-setting processes, organ procurement and

transplantation policy, and the role of science and technology in countering terrorism. Dr. Pope is the recipient of the National Academy of Sciences President's Special Achievement Award and the IOM's Cecil Award.

Sarah L. Hanson is associate program officer for the Forum on Neuroscience and Nervous System Disorders on the Board on Health Sciences Policy at the Institute of Medicine. Ms. Hanson previously worked for the Committee on Sleep Medicine and Research. Prior to joining the Institute of Medicine, she served as research and program assistant at the National Research Center for Women & Families. Ms. Hanson has a B.A. from the University of Kansas with a double major in political science and international studies and a minor in women's studies. She recently completed a post-baccalaureate premed program at the University of Maryland and hopes to attend medical school in the future.

Lora K. Taylor is a senior project assistant for the Board on Health Sciences Policy at the IOM. She has 15 years of experience working in the National Academies. Prior to joining the IOM, she served as the administrative associate for the Report Review Committee and the Division on Life Sciences' Ocean Studies Board. Ms. Taylor has a B.A. from Georgetown University with a double major in psychology and fine arts.