



**The Must-Have Collaborations  
for Successful Drug Development:  
Uniting Patients and Caregivers, Researchers, Industry, and Government  
Regulators at the Table**

**A White Paper Expanding the Panel Discussion at the Global Genes Third Annual  
RARE Patient Advocacy Summit  
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**Executive Summary**

Collaborations among academic and government researchers, pharmaceutical and biotechnology companies, regulatory agencies, and patients and their families are crucial to research and development of therapeutics for rare diseases. Industry, academic and government researchers, regulatory agencies, patients and their caregivers all confront challenges in today's environment for therapeutic development. As a result, the stakeholders are realizing increasing value in collaborations, which can help tackle these challenges.

Panelists at the 2014 RARE Patient Advocacy Summit on “The Must-Have Collaborations for Successful Drug Development: Uniting Patients and Caregivers, Researchers, Industry, and Government Regulators at the Table” discussed the following lessons regarding collaborations on drug and other therapeutics development:

- These collaborations serve to advance research and development, build understanding of each other's perspectives, and develop trust
- Each stakeholder has specific, unique strengths to bring to a collaboration, ensuring that a collaboration should be a true partnership among the participants
- Recognizing this need for partnerships, patient organizations are being brought into collaborations earlier in the research and development process and also increasingly leading collaborations involving many institutions and stakeholders
- However, the need for partnerships also has raised challenges in connecting with partners and managing the different perspectives within the collaboration
- Successful collaborations require strong communication with integration of team members and full sharing of data

These lessons described by the panelists are discussed in detail within this white paper.

**Introduction**

Researchers, industry, regulatory and patient groups all need an equal seat at the table for research and development (R&D) for new drugs and other therapeutics to occur. Historically, this has not always been the case. Patients and their organizations were often not involved in the development process other than as clinical trial participants. Today, due to financial and resource limits and the urgent unmet needs of patients, the process for developing effective therapies is evolving. Many rare disease R&D projects and regulatory decisions are integrating closer collaborative ties among patients, researchers, companies, and regulators -- ties that are now often led by patients and disease advocacy organizations themselves.

At the 2014 RARE Patient Advocacy Summit, Global Genes initiated a discussion among stakeholder representatives to develop insights on collaborations for successful therapeutic development that can be used by rare disease patients, caregivers, and advocacy organizations as well as researchers, industry, and regulators. Panelists were Barbara Wuebbels, Vice President of Patient Advocacy, Audentes Therapeutics; Wendy White, Founder and CEO, Siren Interactive; Jayne Gershkowitz, Vice President, Patient and Professional Advocacy and Public Policy, Amicus Therapeutics; Steve Groft, Pharm.D., former Director, National Institutes of Health (NIH) Office of Rare Diseases Research; Hudson Freeze Ph.D., Professor and Director, Human Genetics Program Sanford Children's Health Research Center, Sanford-Burnham Medical Research Institute; Matt Wilsey, President, Grace Wilsey Foundation; Jonathan Jacoby, Hide & Seek Foundation, Support of Accelerated Research for Niemann Pick C (SOAR-NPC); and Katherine Rauen, M.D., Ph.D., Chief, Division of Genomic Medicine, Department of Pediatrics, University of California Davis Health System and University of California, Davis Medical Investigation of Neurodevelopmental Disorders (MIND) Institute.

As collaborations are on the rise, many efforts are ongoing to understand effective formal organization and management. This stakeholder discussion complements these ongoing efforts and focuses primarily on providing an understanding of:

- The critical role of collaborations in successful therapeutic development
- Each stakeholder's strengths and roles in collaborations
- How collaborations are evolving to include strong patient involvement and diverse leadership
- Challenges of differing stakeholder perspectives
- The critical role of communication in successful collaborations

## **DEFINITIONS**

**Therapeutic:** a treatment for a disease or disorder and may be a drug, device, or process

**Academic researchers:** researchers employed directly by a higher education institution (e.g., the University of California, Stanford University) or non-profit institution (e.g., Scripps Research Institute, Sanford-Burnham Medical Research Institute, Lovelace Respiratory Research Institute)

**Government researchers:** researchers employed directly by a federal or state agency, e.g., the National Institutes of Health, the Department of Defense, the California Institute for Regenerative Medicine

**Pharmaceutical/biotechnology industry:** companies conducting research, development, and marketing of therapies requiring regulatory approval

**Regulator:** an organization that oversees the process to ensure efficacy and safety of

therapeutics, principally the U.S. Food and Drug Administration for therapeutic development in the U.S.

**Stakeholder:** Individuals or organizations that are affected by and/or have influence over the research and development process, i.e., patients and their caregivers, government research and regulatory agencies, academic researchers, and the pharmaceutical industry

**Collaboration:** Joint research or regulatory development effort among more than one stakeholder that could include funding, sharing resources and data, or sharing perspectives on patient medical needs and benefit-risk assessment

### **Critical Role of Collaborations in Successful Therapeutic Development**

Industry, academic and government researchers, regulatory agencies, patients and their caregivers all confront challenges in today's environment for therapeutic development. As a result, the stakeholders are realizing increasing value in collaborations, which can help tackle these challenges.

Today, to bring a drug or other therapeutic to market requires 10 to 20 years of R&D, millions of dollars, and meeting stricter requirements for demonstration of effectiveness while simultaneously facing extensive budget cuts. Barbara Wuebbels also highlighted that therapeutic development for rare diseases faces a number of additional challenges: a small number of patients, setting up multiple clinical testing sites to gather enough data for regulatory agencies which increases the already high cost of drug development, and sales force and distribution challenges to educate and reach patients and physicians spread across the globe.

Academic and government researchers are facing the challenge of steeply declining resources. This not only drives researchers to seek additional funding from other resources such as patient groups, but it also impedes the research process. Hudson Freeze, Ph.D., highlighted the connection between this challenge facing researchers and the difficulties within the entire R&D process: "funding for fundamental research has decreased by 25% in the last 10 years .. the fundamental science may affect your child... and as long as we keep robust funding ... we have a chance."

Regulatory agencies must balance rapid approvals of effective therapies while ensuring that appropriate safety concerns are identified and addressed. However, regulatory agencies are beginning to recognize that patients and their caregivers may value risks and benefits very differently than what has been set as standards by industry and government agencies. This point was emphasized throughout the 2014 RARE Patient Advocacy Summit, particularly by Pat Furlong, President of the Parent Project Muscular Dystrophy. She discussed the changing efforts to assess benefits and risks of therapies in FDA reviews, describing the increasing efforts being made by patient groups to contribute their perspective. She noted that physicians and researchers may value certain improvements much less than patients and caregivers do. For

example, caregivers may strongly value the benefit of a therapy that improves muscle tone enough to allow a child to turn while sleeping, allowing the caregivers to get more sleep rather than being woken by an uncomfortable child. But without the caregiver perspective, regulators and researchers may not recognize this as a strong enough benefit to patients and caregivers worth the risks of a therapy. Ms. Furlong also discussed that patient preferences may shift over time, with the willingness to accept greater risks from a therapy possibly increasing as the disease progresses. The FDA is increasingly trying to incorporate patients' perspectives into the assessment of safety and efficacy in the product development cycle of therapeutics.

At the same time, patients and their caregivers, particularly those with rare diseases, are experiencing urgent and unmet medical needs and are no longer simply waiting for help. All panelists discussed the changes brought through knowledge gained by patients and their caregivers vigorously following research and by connections strengthened by social media. Patients have found greater empowerment within the development process and are actively seeking therapies and forcing other stakeholders to rethink research priorities and preferred outcomes. They want to be included earlier in the research process, to set goals and priorities, to raise funding, and to keep all stakeholders focused on the mission of rapidly developing effective treatments. Dr. Freeze described it as more strongly uniting researchers and patients, "our labs are only a click away from all of you." Many pharmaceutical and biotechnology companies have instituted a patient relations or patient advocacy role within the company to facilitate collaborations with patients and serve as a single point of contact for any questions on R&D. Jayne Gershkowitz described her role as "help[ing] patients to understand what the reality is in terms of legality and timeframes". She urged patients and caregivers to approach patient advocates at companies when they are seeking more information on current research efforts.

Within this environment, all stakeholders can greatly benefit from each other and must figure out how to work together.

### **Stakeholder Strengths and Roles**

Each stakeholder in the R&D process fulfills specific roles and brings specific strengths.

#### *Strengths and Roles of Academic and Government Researchers*

Panelists discussed that academic and government agency researchers bring the ability to conduct excellent research and analysis, often in combination with an understanding of the clinical symptoms and progression of the disease through interactions with patients in a clinical setting. These strengths can be used both in developing basic and clinical research projects as well as working with patient and industry organizations to prioritize projects. Government agencies also provide public funding resources in support of basic and clinical research efforts.

#### *Strengths and Roles of the Pharmaceutical/Biotechnology Industry*

Pharmaceutical and biotechnology companies contribute research expertise and private funding resources to collaborations. Industry also provides the expertise to take a therapeutic through the clinical testing and regulatory approval process. They educate physicians on the use of the product and are responsible for the registration and distribution of the product across multiple countries.

### *Strengths and Roles of Regulatory Agencies*

Regulatory agencies also contribute scientific and medical expertise to evaluate safety and efficacy.

### *Strengths and Roles of Patients and Caregivers*

Patients and their caregivers have always brought the most important aspect: the recognition of medical needs and the passion to seek, and encourage others to seek, ways to improve the symptoms and slow or halt disease progression. Patients and advocacy groups have a wide and varied set of strengths to use. Matt Wilsey clearly emphasized that, most importantly, patients bring passion, determination, courage, and strength of commitment as the top stakeholder in the effort to address rare diseases. Many of the panelists also noted that patients and their caregivers bring extensive medical insight – they live with the symptoms and progression of the disease. Because of this, they can contribute valuable medical observations to researchers and an understanding of the priorities of patients to industry and regulatory agencies. Patients also bring their own skills to the fight against rare disease developed through their professional and personal lives. Jonathan Jacoby provided just a few examples of these skills including fund raising, communications, project management, or risk-benefit assessment. Finally, patient organizations can provide needed resources including money and access to patients to support research and also widely shared research resources such as cell colonies, biospecimen banks, or animal disease models.

### **Evolving Patient Participation and Roles in the Collaborative Research Model**

Until recently, stakeholders in the rare disease therapeutic development process were largely operating with minimal collaboration. Patients and their caregivers have recently developed new roles with more intense and focused collaborations with researchers, industry, and regulatory agencies. They are bringing their strengths as active participants to all aspects of the R&D process: early-stage research, clinical research, regulatory policy-making and therapeutic approvals, and continued monitoring of the safety and efficacy of therapies. Stakeholders have realized the key role of close collaboration with patients and other stakeholders to successful therapeutic development.

Getting new therapies to patients requires the involvement of:

- academic and government researchers to develop an understanding of the causation and natural history of disease and identify possible therapeutics
- pharmaceutical and biotechnology companies to develop and test the therapeutics through clinical research and regulatory approval and then manufacture and sell the therapeutics
- government regulators to ensure safety and efficacy, and
- patients and their caregivers to participate in the entire research process: identifying treatment priorities, focusing research efforts, and funding basic and clinical research

Greater and earlier participation of patients may help speed and focus the R&D process, getting therapeutics to patients faster. Although all stakeholders are needed to take a therapeutic from

the research lab to the patient, the process may involve multiple individual collaborations among the stakeholders. In collaborations such as advisory boards and research collaborations, industry and regulators are seeking patient input earlier and more extensively on clinical trial design, prioritization of unmet medical needs, risk-benefit assessment, and even product labeling and communications to patient groups. Patients and advocacy groups have also begun to actively develop and manage research collaborations. They seek out the best researchers; assist with identifying, prioritizing, and funding research projects; evaluate research that their organizations have helped fund; develop and provide research resources; and even start companies to develop and market therapeutics. Rather than collaborations being led by industry with limited involvement by other stakeholders, research collaborations now are driven by all participants. Academics may seek out industrial and patient advocacy partners for research. Likewise, industry develops and leads collaborations with patients and caregivers and academics. Regulators and government agencies develop collaborations as advisors on regulatory policy and as research partners with academics, industry, and patients. Patients and advocacy groups also now develop and lead collaborations closely involving all stakeholders. Ultimately, there is a synergy created that does not exist without close collaboration among all stakeholders and close, continued involvement of patients.

### **Challenges for Successful Collaborations**

Collaborative efforts are not easy. Successful collaborations must manage and overcome two principal challenges:

- Connecting with other stakeholders
- Differing perspectives of stakeholders

#### *Connecting Stakeholders*

Collaboration first requires finding other stakeholders willing to participate. Although there are some formal, organized channels, many of the connections are made through informal means.

Formal mechanisms include government and industrial advisory boards. The U.S. Food and Drug Administration (FDA) and the National Institutes of Health (NIH) have many advisory boards that include one or more roles for patient and caregiver advocates to “bring the voice of the patient” to the agency. Patient Representatives can apply with the FDA to serve as voting members on advisory committees to provide the patient perspective early in the regulatory review process (see “Additional Resources”). The NIH also has funded consortia through universities that bring together researchers, patient groups, and industry. Steve Groft, Pharm.D., described the goals of these NIH-funded consortia as “bringing patient and research communities together so the patients understand research better and the researchers can get the perspectives of the patients.”

Many companies also seek patient participation on advisory boards, sometimes through formal requests and sometimes through more informal means. These advisory boards provide input to industry researchers on what outcomes patients value, some of which researchers and regulators may be discounting (e.g., Ms. Furlong gave the example that patients may view an outcome of improved sleep as valuable which may not be considered by others), how to communicate with the patient population, what the risk-benefit assessment of patients and their caregivers is (e.g.,

another example described by Ms. Furlong is that some patients and caregivers may accept a higher risk of adverse events as the disease progresses), and the potential difficulties of the research plan for patients (e.g., Dr. Groft described potential issues such as can patients adhere to the schedule of the clinical trial, is the informed consent form understandable, is the time or travel asked of patients too much). Often participants for these advisory boards are found through requests by the industrial patient advocacy office or liaison to patient advocacy organizations.

Connections between academic and industry researchers can occur through formal mechanisms, often a company licensing a technology from a university. However, often connections are made through scientific meetings or introductions through other researchers or patient advocates.

Connections between patients/caregivers and academic and industry researchers are also made through both formal and informal mechanisms. Formal mechanisms include the new model of venture philanthropy where patient advocacy organizations award research grants through a formal application process. Most connections are built slowly. Both Mr. Jacoby and Mr. Wilsey noted that connections are built relationship by relationship over time. Patient advocates and researchers recommend personal contacts through email or phone calls. Especially important is to remember that sincerity and follow-up effort are critical. Mr. Wilsey summarized the process as, “a sincere email goes a long way, but there must be continual follow-up -- there is no magical wand, and it requires a lot of elbow grease.”

#### *Managing Different Stakeholder Perspectives*

The second principal challenge cited regarding collaborations is managing the different perspectives of the stakeholders. Although all stakeholders have the ultimate goal of improving outcomes for patients, they do all also have their own goals and timelines. These conflicts can be managed implicitly through recognition and acceptance or explicitly by addressing them head-on.

Conflicts that cannot be readily changed can be recognized and accepted as part of the collaboration. Commonly cited stakeholder goals that stand in conflict with each other are:

- The need for industry to receive a financial profit for therapeutics,
- The need for the FDA to successfully protect the public from unacceptable risks, balanced against
- The need for patients and caregivers to have as fast a development timeline as possible

Other stakeholder perspectives can be explicitly addressed within the structure of the collaboration:

- The professional requirement that academics must publish journal articles and support research through successful grant applications
- The reluctance of academic and industry researchers to share information and data
- Intellectual property concerns
- Academic researchers focusing efforts where funding is readily available

For example, collaborations should be structured so that academic researchers feel confident they can develop publications and use the data for future grant applications. Katherine Rauen, M.D., Ph.D., also highlighted that many patients can and should be involved in the publication process -- in fact, she asked this question to the audience of patient advocates and about one-third indicated they had been listed as a co-author on a scientific publication. With the help of academic and government researchers, collaborations can also involve research efforts that can be used as part of a graduate student dissertation or thesis. Collaborations can be structured so that participation requires an agreement that data and resources will be shared. Mr. Wilsey and Mr. Jacoby emphasized the importance of sharing data for moving the research forward. For their organizations, it is a requirement of their funding that researchers share the data at least within the team of researchers funded by their organization: “share early and share often” is how Mr. Wilsey described the requirement. Intellectual property agreements can be negotiated at the start of the collaboration, drawing on templates already developed. For example, Support of Accelerated Research for Niemann-Pick Disease (SOAR-NPC) has developed intellectual property templates that could be used. Finally, collaborations can be structured to include financial and resource support to encourage academic researchers’ efforts on a topic.

### **Characteristics of Successful Collaborations**

Quality, effective communication about the goals of the collaboration and the goals of the stakeholders is the critical key to successful collaborations. Collaborations involving research efforts should be set out with measurable and reasonable milestones and regular evaluations at the start of the collaboration. Again, this is a requirement of researchers funded by the Grace Wilsey Foundation and the SOAR-NPC organization. Sharing of data and information should be made as easy as possible. For example, collaborations should consider setting up a secure solution to allow for rapid data sharing. Stanford University and the Grace Wilsey Foundation developed a secure solution so that researchers could share quickly at the request of the NIH. Although ongoing communication should be encouraged, face-to-face meetings are invaluable and should be scheduled regularly. Scientists must be ready to speak about the disease and research efforts in terms that are understandable to the patients/caregivers and within larger communication efforts such as the news media. Stakeholders must respect each other’s goals and needs. Finally, the collaboration participants should actively strive to continue to develop connections through meetings, phone calls, and emails.

### **Conclusion**

The key role of collaborations in the R&D process is recognized by all stakeholders. Collaborations also have been evolving over the last several years to include patients and caregivers at earlier stages and as more active participants and to include collaborations initiated and led by all stakeholders. With this evolving nature of collaborations, efforts to understand both the formal management and the informal aspects are critical. This stakeholder discussion provides valuable lessons about the critical role of collaborations in successful therapeutic development, the stakeholder strengths, evolving patient roles in collaborations, the challenges of collaborations, and the characteristics of successful collaborations.

### **Case Studies of Collaborations Involving Patient/Caregiver Participation**

Collaborations are evolving to include patients and their caregivers at earlier stages and to include leaders from all stakeholders: industry, academic/government researchers, regulatory agencies, and patients. These collaborations have a primary goal of facilitating R&D efforts, but they also serve to build relationships and trust among the stakeholder groups. The following appendices provide a very brief overview of examples of collaborations led by each stakeholder group described at the RARE Patient Advocacy Summit or by the panelists in follow-up discussions.

### **Collaborations Led by Industry**

Patient advisory boards are one example of a collaboration that is led by industrial representatives. Barbara Wuebbels of Audentes Therapeutics discussed the patient advisory boards hosted by her company. Interest in participation in these advisory boards has been growing. Patients or their caregivers quickly volunteer to participate, which often involves time away from their family and overnight travel. The companies employ patient advisory boards to understand what patients want and how to meet their needs, e.g., what benefits do they want from therapeutics, what are acceptable risks. Through patient advisory groups, the company can also provide realistic expectations to patient groups, letting them know a timeline for the research process and when the therapeutic might be available commercially. Ms. Wuebbels summarized this goal of the advisory groups as “this is an excellent opportunity to bring the voice of the patients to the company and the voice of the company to the patients.”

### **Collaborations Led by Academic/Government Researchers**

The NIH, through the National Center for Advancing Translational Sciences (NCATS) Office of Rare Diseases Research and many of its other institutes, have funded collaborations through the Rare Disease Clinical Network (RDCN). The RDCN was started in 2003, and 22 consortia were funded in this past year. The goal is to advance research on rare diseases by facilitating (1) collaborations among researchers from several fields, patients, and industry; (2) data sharing; and (3) clinical trial recruitment and enrollment. Each consortium must include involvement of patient advocacy groups and research on at least three related rare diseases. Each consortium is led by an academic researcher who serves as the principal investigator, but they have monthly meetings with the patient groups who set the agenda in consultation with a program manager at NIH.

Dr. Graft described the RDCN as an effort to build “a critical mass of investigators” from multiple fields and multiple locations to ensure a full approach to rare disease research and a critical mass of patients so that clinical studies can be completed as quickly as possible. He also noted that these collaborations serve to build trust and increase the understanding of the patient experience, the researcher experience, and the company experience in getting a therapeutic to market among the participants.

### **Collaborations Led by Regulatory Agencies**

The FDA leads the Patient Representative Program, which aims to ensure patients’ perspectives are included in therapeutic approval and policy decisions. Patients or caregivers serve as a member, often as a full voting member, on advisory and review committees or provide presentations at FDA meetings.

## **Collaborations of Patients, Caregivers, Advocates, and Scientists**

### **1. Support of Accelerated Research for Niemann-Pick C Disease (SOAR-NPC)**

SOAR-NPC was created by a group of parents, advocates, and scientists in 2007-2008 to provide a scientist-clinician-parent research group. NPC is a rare lysosomal storage disease that results in progressive neurological damage and death from related complications as a teenager or young adult.

Mr. Jacoby described it as “growing out of a common understanding among a group of scientists and parents that lay roles could be more useful than understood in the past.” NIH, in close coordination with SOAR-NPC and other NPC foundations, is now conducting a clinical trial of cyclodextrin. Cyclodextrin is an example of a potential therapeutic application for a chemical already used for other applications – cyclodextrin is used as a stabilizing agent in the food industry and in the drug manufacturing process. Current participants include researchers and clinicians from NIH, academic researchers, regulators from the FDA, researchers from Johnson & Johnson who are providing cyclodextrin, and parent groups.

SOAR-NPC has a core scientific research group. These researchers submit annual proposals, discuss the research plans with the parent groups, and then revise the plans as needed. The organization also funds small pilot projects for researchers outside the core group.

Mr. Jacoby noted that it is important to provide incentives to researchers that are at least as strong as the others they are facing and to find collaborative researchers: “We found a group of scientists and made sure they 1. knew the kids -- that they had interactions with kids with NPC-- and 2. they had a collaborative inclination -- they were willing to share information and they knew that had to be beyond sharing an abstract at a conference. But how do we get them to collaborate? The answer was money. But it is important to know that you don’t have to raise all the money an investigator needs. You can incentivize someone and then leverage your money so you get more out of it.”

Mr. Jacoby also noted that it is important to be sure that decisions in these collaborative organizations are made with strong input from those with the greatest knowledge of the patient needs: “It is really valuable to think in anti-hierarchical fashion because when you think in hierarchical fashion then patients are at the bottom, then scientists, then the scientific advisory committee, and then funders. So decisions about what to do were almost always made by the ones not in the trenches. The ones who should be making the decisions are the patients and the scientists. So parent-funders in SOAR meet two to three times a year and have done away with the idea that there should be some higher body that makes decisions about where money should go. This really helped accelerate the process so there is very little bureaucracy -- it’s mostly about what the scientists say they can do. We have very clear milestones. If the scientists don’t achieve the milestones, they are the first to say let’s try something else.”

But ultimately, the greatest incentive to emphasize is helping the patients: “We are all held together by a sense of we know why we’re doing this -- we are doing this for those children. We are always measuring our success by what’s being accomplished for them.”

## 2. Grace Wilsey Foundation

The Grace Wilsey Foundation was recently created to fund research for NGLY-1 deficiency. Children with a mutation in the NGLY-1 gene do not produce sufficient amounts of an enzyme to degrade a protein, which then leads to cell damage.

Building on approaches used by earlier organizations such as SOAR, recent efforts by patient organizations are often trying to move faster and create a stronger leadership role for the patient organization. Matt Wilsey is Grace's father, and his full-time job now is applying a Silicon Valley-business model to what he is doing with the Grace Wilsey Foundation: sharing data early and often, moving fast, providing substantial seed capital, and focusing the team on objectives.

Mr. Wilsey strongly believes that the combination of patients with multiple academic researchers and private companies is critical to rare disease research and development. The Grace Wilsey Foundation is serving as the lead to fund and manage multiple research projects. Mr. Wilsey developed these relationships with researchers and companies "one by one" -- he found one trusted source and asked for other experts and knowledge leaders. Then via cold email and calls he recruited a handful of trusted sources who could advise him. His mission is to identify gaps in the current research efforts, find the best researchers to address those gaps by seeing which names keep coming up in discussions, and then reach out to them to see if they would be amenable to working with the Foundation's team. Mr. Wilsey cold calls or sends concise email requests that relate to a researcher's previous role. Most of the time this generates a favorable response. However, it requires constant follow-up: "Getting momentum is hard; it takes a lot of follow-up. You have to stay on people's radar and follow-up. It's also imperative that patients or advocates make fast funding decisions and let researchers know why they might not receive funding at this time."

Each time he adds a new researcher to the team he circles back to all the other people and ensures that the researchers are well integrated with each other. All research projects undergo regular evaluation, assessing whether milestones are being met and providing quarterly reports of progress.

Mr. Wilsey notes that it is not a natural instinct to collaborate, especially given reduced government funding for life sciences: academia is focused on writing grants for research they have already accomplished to a large degree, researchers are looking for areas that are getting a lot of funding, and companies are not collaborating. Mr. Wilsey specifically looks for researchers who are willing to be collaborative, but also plays a role in encouraging collaboration and sharing.

Ultimately, Mr. Wilsey finds that patient advocacy efforts and private financing can help to get academic researchers working on something where they feel part of something special: "Researchers could work on lots of valuable projects but they work on issues in part because of the passion and determination of patients and their families."

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