The Multiple Myeloma Research Consortium:

Building a home for an orphan disease

PRICEWATERHOUSECOOPERS

In 1996, Kathy Giusti had it all: A loving husband, a young family and a flourishing career as a pharmaceutical executive. Then came a phone call that changed her life: Her doctor told her she had cancer.

The diagnosis was multiple myeloma, a rare form of blood cancer that damages bone marrow cells, often leading to anemia, decreased immune function and renal failure. By the time the disease is diagnosed, most patients have myeloma cells in multiple sites throughout their bone marrow. Kathy was given only three years to live.

Still reeling from the news, Giusti began learning all she could about her disease. What she found was devastating: Multiple myeloma is incurable—and invariably fatal. Because it accounts for only about one percent of all cancer diagnoses, it is an "orphan" disease, generating relatively little research interest and funding. While several research programs had made inroads toward treatment, their efforts were fragmented; there was ad hoc collaboration among some researchers, but no formal coordination of research or sharing of ideas, experimental data or clinical trial results.

Giusti saw the lack of collaboration among researchers as a barrier to the discovery and development of treatments for myeloma. Accustomed to aggressively tackling challenges in her business career, she was determined to accelerate the search for a cure.

Accelerating the search for a cure

Giusti established the Multiple Myeloma Research Consortium (MMRC) in 2004. She envisioned the MMRC bringing together leading academic institutions to conduct collaborative research in three key areas of myeloma research: Genomics, target validation and clinical trials. Four prestigious cancer research centers signed on: The Dana-Farber Cancer Institute, The Mayo Clinic, The H. Lee Moffitt Cancer Research Institute and Princess Margaret Hospital at the University of Toronto.

Bringing together such a disparate group of researchers and institutions was challenging. Most research consortia dissolve quickly due to a lack of interest, diverging priorities or poor organization. With no cure or breakthrough treatment on the horizon, the consortium Giusti envisioned would have to be sustainable indefinitely.

Based on the recommendation of Dr. Jeffrey Trent, president of the Translational Genomics Research Institute (T-Gen), Giusti asked PricewaterhouseCoopers (PwC) to help organize the consortium. PwC's Growth and Innovation practice had helped to create T-Gen, a $60 million biomedical research institute. An "open innovation" model that the Growth and Innovation practice uses to link complementary networks to produce innovations that no single organization could achieve on its own seemed to be an effective way to draw together the widely-dispersed researchers working on myeloma.

Developing guiding principles

A first crucial step in PwC's work in organizing the MMRC was to help develop operating principles that would unite myeloma researchers in a sustainable collaboration. For Giusti, speed-to-market was the number-one priority: Academic credit, ownership of intellectual property and other thorny issues would have to take a back seat to the goal of finding a cure as quickly as possible. Based on those priorities and on extended conversations with the Consortium's original members, the PwC team articulated six key operating principles:

- **Collaboration:** A willingness to forego individual gains, at least temporarily, in favor of collective efforts and discoveries
- **Urgency:** A shared determination to find a cure as quickly as possible
- **Excellence:** A desire to engage global leaders in the field of myeloma research and drive new discoveries by combining their expertise with leading techniques and technologies
- **Focus:** A commitment from the scientists to make Consortium research a priority
- **Publications and sharing of academic credit:** An appreciation of the need to reward hard work and foster participation by younger investigators
- **External focus:** An acknowledgement that other institutions, communities and disease groups can provide valuable insight into the search for a cure

Designing the organizational structure

The next challenge was creating an organizational blueprint for the MMRC. The Consortium's research would have to be organized in a manner that would enable it to accomplish its aggressive scientific goals while generating the greatest impact from its investment.

"Based on interviews with officials of academic medical centers, leading myeloma researchers and representatives from other research consortia and discussions with Kathy Giusti, we concluded that a bricks-and-mortar organization was too costly and not the best way to spend limited research dollars," says Gerry McDougall, the PwC partner who led the engagement. "We also recognized that a purely virtual organization would not be able to provide adequate resources or to direct the efforts of the Consortium."

The solution was a hybrid organization with a central hub, central management and central operating principles and parameters, but with virtual core laboratories that would leverage the distinctive expertise of each member institution.

Under this hybrid organizational model, three multi-site core laboratories spread among a number of institutions were established, enabling MMRC to leverage existing expertise and resources and avoid the need for costly (and likely duplicative) investments. "Rather than reinvent the wheel, we leveraged other people's wheels," says Bill Dracos, the PwC professional who was the lead manager on the engagement. "Instead of building core labs, we are using labs that already operate very well at various universities and which also happen to focus on myeloma." Leading myeloma researchers direct each core, working under the auspices of their home organizations, with a coordinating committee overseeing their work.

This organizational structure, combining central resources and operating principles with a large measure of institutional autonomy, had never before been tried. It was created to meet the needs of the myeloma researchers and their institutions—and to support Kathy Giusti's mission of removing the scientific obstacles to a cure.

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Tissue and data banks

In addition to the core laboratories, the MMRC developed a tissue bank which serves as a repository of patient samples and fuel research. The future of the tissue bank would depend on building a reliable mechanism for tissue transport and tracking. With that in mind, the PwC team helped to devise a system in which a central location would receive tissue samples, process them according to a standard protocol, store them and redistribute them to the core laboratories.

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Winning the buy-in of researchers

In discussions with researchers who would be involved in the Consortium, it became clear that it was crucial to balance the medical research community's inherent competitiveness with the need for better collaboration. "One of the success stories of the Consortium is that leading researchers had the vision to see a bigger goal and to envision better results in the long term by collaborating than by working in relative isolation," says Dracos. The interviews confirmed the importance of keeping the ultimate goal of a cure first and foremost.

The interviews also informed the development of the Consortium's research funding mechanism. They confirmed that, in academic medicine, researchers follow the funding. Accordingly, the Consortium provides strong funding in areas it believes to be the most promising or where fundamental obstacles need to be overcome. To ensure the quality of research, the Consortium does not simply dole out money. Rather, it requires researchers to compete for grants in a process with the same quality standards demanded of federally-funded programs. This rigorous process also assuaged concerns that a single institution would come to dominate the Consortium, and ensured that the researchers would work as equal partners.

Clearing the intellectual property hurdle

The institutions did not always share their researchers' enthusiasm for participating in the MMRC. "Our goal was to pull this Consortium together to drive research and cures, and the goal of the universities was to protect their intellectual property," says McDougall. "The MMRC was less concerned with ownership of intellectual property than with getting promising treatments to market. The Consortium was willing to compromise in order to keep moving toward achievement of its long-term vision."

PwC worked closely with the MMRC and its legal counsel to create affiliation, intellectual property and materials transfer agreements that balance the intellectual property rights of the member institutions with the drive for a cure.

Under the agreement, the Consortium respects the participating organizations' intellectual property policies; however, if discoveries are made using MMRC funding, a portion of the intellectual property rights will revert to the Consortium. In addition, the MMRC negotiated a "right to market" clause, which stipulates that discoveries cannot be "shelved"; if a participating institution chooses not to go to market with its intellectual property, the MMRC has the right to do so.

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Defying the odds

PricewaterhouseCoopers spent more than a year helping Kathy Giusti to realize her vision of uniting myeloma researchers in the quest for a cure. Today, the MMRC is directing research on a disease that, while still incurable, is no longer an orphan. The Consortium now has 13 participating and member institutions and expects to add other leading research organizations.

Soliciting the input and buy-in of researchers early on, and building a sound organizational structure, have proven to be key factors in the Consortium's success. Today, the MMRC is an enterprise that continues to work towards a shared long-term goal. MMRC executives and lead researchers meet at least quarterly to evaluate their progress, and the Consortium remains willing to change its structures, make accommodations and resolve conflicts to accelerate progress.

The MMRC has attracted significant attention to myeloma, and its targeted research has made it a magnet for fundraising. Donors know that their money is going to specific research projects directed toward achieving clinical results, and this has helped the MMRC to raise $92.4 million to further myeloma research.

Those funds have helped the Consortium to make considerable progress in the four years since its founding. Although it typically takes a decade to develop a drug and have it approved, MMRC researchers have already won approval of four new drugs. The Consortium currently has eight clinical trials in progress, seven projects under evaluation and 30 drugs or drug combinations ("cocktails") in development.

The MMRC is achieving its short-term goal of developing rapid, cooperative and innovative approaches toward the development of therapies for myeloma. And it is on a path toward realizing a long-term objective—facilitating the development of individualized treatments, using genetic markers in patients to direct targeted therapies. Researchers already have identified several promising genomic targets.

As for Kathy Giusti, the visionary behind the MMRC continues to defy the odds, refusing to let her own disease interfere with her work toward a cure. With her twin sister Karen Andrews, she founded a sister organization to the MMRC, the Multiple Myeloma Research Foundation, and continues to raise awareness of myeloma and support for the development of a cure. And, 12 years after being told she had no more than three years to live, her cancer is in remission.

Contacts

Gerald J. McDougall
PricewaterhouseCoopers
Global Health Sciences Leader
(617) 530 4471
gerald.j.mcdougall@us.pwc.com

Bill Dracos
PricewaterhouseCoopers
Director, Health Industries Advisory Services
(678) 419 1591
bill.dracos@us.pwc.com

pwc.com/healthcare
pwc.com/hri