

# Patient Advocacy

An emerging role within pharma and biotechnology

White Paper Presented by BioNJ and Merrill DataSite®



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Dear Reader,

As the life science industry continues to advance the discovery and development of new therapies and cures, research-based companies are faced with tackling even more complex and difficult-to-treat diseases. As part of their efforts, companies are beginning to recognize the value of bringing together researchers, Patients, caregivers and advocacy groups in a unified and comprehensive way to meet a common goal.

Developing these relationships among all these parties is vital for advancing drug discovery and development—because Patients can't wait. And that's why we are so pleased to present this white paper—Patient Advocacy: An emerging role within pharma and biotechnology.

The white paper is based on a survey that was conducted by Merrill DataSite in June of 2012 and the BioNJ webinar Helping Companies Achieve a High Return on Investment in Patient Advocacy Programs held on December 19, 2012.

As reported in the white paper, many companies are at the forefront of developing programs to work with patient advocacy proponents because of their shared interest in helping patients. Collaboration is important during all stages of the drug development process ranging from raising funds for research, to designing clinical trials, to interacting with the U.S. Food and Drug Administration. The purpose of this white paper is to provide companies with resources and best practices for building patient advocacy functions within their corporate structures.

We have many people to thank for all of their efforts and hard work in moving this initiative forward and developing what we hope is the first of many resources on this very important topic.

We are very thankful to the BioNJ Patient Advocacy Committee, comprised of Amicus Therapeutics, Celgene Corporation, ImClone Systems Corporation, a wholly-owned subsidiary of Eli Lilly and Company, PTC Therapeutics and Sanofi, for their guidance on this initiative. We must also thank our webinar panelists and moderator for participating and sharing their keen insights and experiences during the webinar as well as all those who participated in both the survey and the webinar.

And finally we must extend a huge thank you to Merrill DataSite for partnering with BioNJ on this very important initiative, for conducting the survey and developing this valuable resource.

We hope you find this white paper to be beneficial as you look to grow your patient advocacy function and ensure that the voice of the Patient is heard.

Yours in BioNJ,

Francois Nader, M.D.  
BioNJ Board Chairman  
President and CEO  
NPS Pharmaceuticals, Inc.

Debbie Hart  
President and CEO  
BioNJ

Many biopharmaceutical companies have established strategic patient advocacy functions within their corporate structure. Patient advocacy functions foster relationships with patients, their caregivers, and the disease-specific nonprofit advocacy groups that support them. These interactions ensure the voice of the patient is understood across every other function within the company, from R&D to commercialization.

Within the company, patient advocacy helps personalize a rare disease or disorder, allows companies to focus on unmet medical needs, can help inform clinical trial development, and create a feedback loop with patients about their experience with the therapy.

To help facilitate the role of the patient advocacy professionals at member companies, BioNJ is partnering with Merrill DataSite® to develop a sustainable initiative, providing industry resources and best practices. At the launch of the initiative, BioNJ and Merrill conducted a member survey and hosted a webinar on patient advocacy.

A total of 75 biopharmaceutical companies in New Jersey participated in the survey, representing a broad diversity across the industry, from companies with five products in development and more than \$500 million in revenue to those with no revenue.

Understandably, those companies had different levels of engagement with patient advocacy, and provided vastly different definitions of “patient advocacy”. However, there was significant agreement, allowing the webinar to begin with this definition:

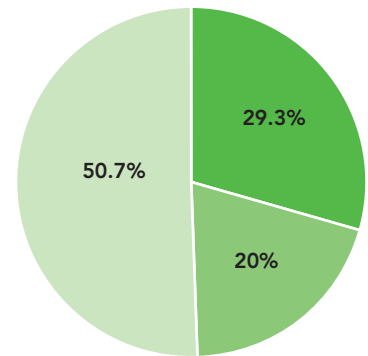
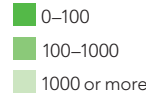
**Patient Advocacy:** A strategic function within the company that serves as a connection point between the patient community and the company, serving to raise awareness of a disorder and possible therapies, facilitating a free flow of information, and helping to discover and address patients’ unmet needs.

## Trends in corporate patient advocacy

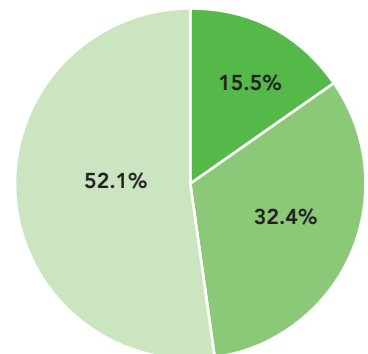
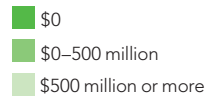
According to the survey, fully 76% of respondents have either established, or are considering establishing, patient advocacy programs within their companies.

However, only 19% of respondents said they had dedicated staff to patient advocacy, showing that while such functions are growing in importance, the resources to staff them are still limited. In fact, while 39% of respondents said their patient advocacy budget is greater than \$200,000, equally

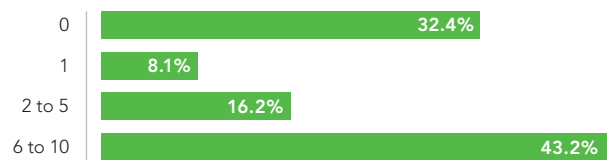
Number of employees



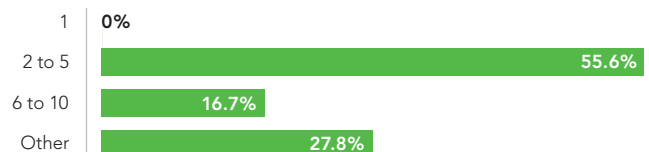
Annual revenue



How many products does your company currently have in the market?

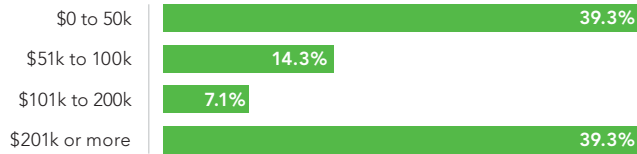


How many products does your company currently have in development?



39% said their budget was less than \$50,000, with the remaining respondents spending an amount in between.

What is the annual patient advocacy budget (primarily referring to donations, corporate contributions, sponsorships and/or educational materials) of the patient advocacy decision-maker?



Among all the respondents, the largest therapeutic area of interest is in oncology (with 60% of respondents focused on it), followed by rare diseases (defined in the U.S. as any disease or condition that affects fewer than 200,000 people) at 40% of respondents.

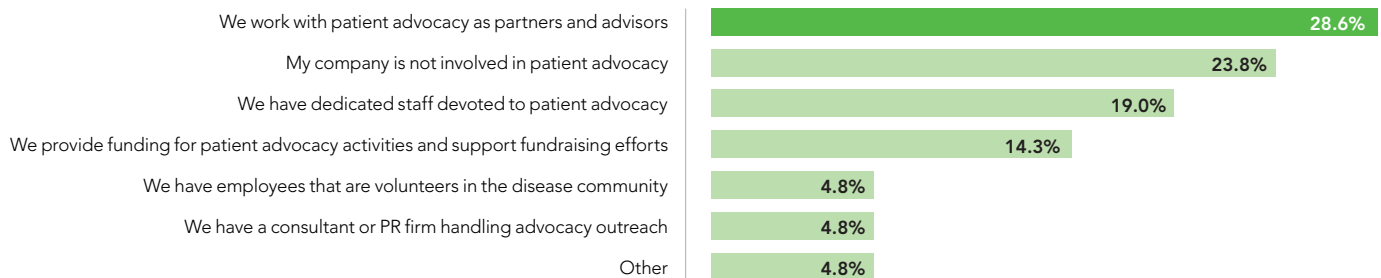
### The role of the patient advocate within the company

As Diane Goetz explained during the webinar, the role of the corporate patient advocate is “being the face of the company to the patient advocacy organizations and to individual patients, and also being the voice of patients within the company so that we always keep patient needs at the forefront in everything that we do.”

The role of the patient advocacy professional is both broad and deep, and can vary widely across companies. Common roles described by the survey include working with nonprofit patient advocacy groups on understanding research and raising awareness (29% of respondents) and providing funding to such organizations (14% of respondents).

Also, the survey revealed that 46% of respondents invite patients to visit the company for educational purposes, often to teach employees about the challenges of living with the disease, inspire scientists and other colleagues who typically do not interact with the patient community.

### What is the primary type of patient advocacy your company is currently practicing?



### BioNJ patient advocacy webinar

#### Moderator:

- **Jean Campbell**  
*JF Campbell Consultants*

#### Panelists:

- **Jayne Gershkowitz**  
Vice President, Patient Advocacy & Public Policy  
*Amicus Therapeutics*
- **Diane Goetz**  
Senior Director of Patient & Professional Advocacy  
*PTC Therapeutics*
- **Cara Thompson**  
Director, Patient Advocacy  
*Celgene Corporation*

At other companies, patient advocacy professionals are directly involved in guiding scientific research, developing clinical protocols, interacting with healthcare providers, and raising public awareness of the disease.

Patient advocacy functions within companies are typically staffed leanly, sometimes by as few as one or two professionals with backgrounds in nonprofit advocacy or healthcare. The job requires frequent travel to medical and scientific seminars to maintain current knowledge of the disease and interact with opinion leaders. Patient advocacy professionals also travel to conferences and meetings hosted by patient advocacy organizations.

Patient advocates in industry are in the unique position to be able to communicate and interact with patients—within company compliance guidelines—and by doing so, gain key insights into the patient experience, and bring that insight within the company, providing essential feedback to the scientific development and clinical trial program efforts.

As Jayne Gershkowitz put it, “It’s our responsibility to develop and nurture trusted and transparent relationships with the patient community, with the individual patients and their families, and then always to represent the patients’ perspective within the company ... cross-functionally and through internal education.”

## Benefits of a patient advocacy function

As the survey illustrated, companies identify many benefits to establishing a strategic patient advocacy function, although respondents noted it might not impact the bottom line directly. While 31% of respondents reported their companies saw a high return to investing in patient advocacy, another 34% said that while the investment was necessary, the direct return was “low”.

However, most companies agree that patient advocacy is so effective because bringing in the patient perspective provides benefits to nearly every other function within the company. It also leads to new possibilities in therapy development that are hard to provide through other avenues.

“Advocacy is not a marketing program, in that the benefits can’t be measured in traditional ways. You can’t look at ROI,” said Goetz. “We can learn from each other, and inform our decisions and grow in a relationship together.”

### Benefits to the company

- Guiding development to unmet needs
- Inspiring and motivating scientists
- Focusing investment on improving patient experience
- Defining clinical trials, raising awareness of new trials and encouraging participation
- Understanding and managing patient expectations for the therapy
- Creating a feedback loop after launch to improve development of next-generation products and to refine patient support services

**In the development stage,** relationships with patients and advocacy groups can provide a unique viewpoint into the phenomenology of the disease.

Gershkowitz said one of the rewarding aspects of her role is “bringing individual patients, families, caregivers and organization leaders in to our company, so that, across our organization, people can really get a comprehensive understanding of the disease experience.”

Being able to interact directly with patients and their advocates provides the company’s scientists with “a complete understanding of [...] what the unmet medical needs are, to inform every aspect of science, program management, and clinical development.”

In this sense, the role of the patient advocate is to facilitate listening, which can have practical benefits and be very inspirational to product development teams. “Our scientists find it so motivating to hear from a patient,” said Goetz. “They are in a lab working on a very small piece of the big picture, and they don’t get to see the end result of their work until [then].”

Goetz provided an example of inviting mothers of patients to video chat with the company’s directors during a board meeting. “It made a strong impression on the Board to hear this first-hand, and it definitely influenced their decision about where to put the next dollar.”

**In the clinical trial stage,** relationships with patient advocacy organizations can raise awareness of new trials, help patients decide whether to participate, and make the risk/benefit tradeoff of the therapy clear to potential patients. By facilitating partnerships with nonprofit organizations the company’s patient advocates can bring the patient perspective to key program decisions, such as setting the eligibility criteria for trials and determining clinical endpoints.

**In the launch phase,** those relationships allow the company to receive feedback directly on how the therapy is working. Once the company’s therapy has been approved, Cara Thompson said, “You’re trying to understand how, where, and why your product fits for a patient and what their needs might be.”

The launch phase “is really a critical time because we’re still gathering a lot of insight on the disease and how people are perceiving things,” said Thompson. “What are the challenges and side effects that we should be focused on and how can we make the patient experience better?”

Relationships between nonprofit groups and the biopharmaceutical community have the potential to be mutually beneficial.

**For patient advocacy organizations**, the relationship provides access to more resources, the latest information on new therapies, and expert insight for their members.

Patient advocacy professionals within industry should make themselves available to nonprofit organizations, to answer questions, provide resources and – when requested – can provide resources and educational grants through the company’s financial support mechanisms.

Common compliance guidelines hold that biopharmaceutical companies should not solicit patient advocacy organizations for providing financial support or informational resources. However, companies can – and should – respond to requests from organizations.

“In oncology,” said Thompson, “treatment approaches and patient outcomes are changing rapidly and these advances bring benefit across the spectrum of patient advocacy organization activities, because the more treatable a disease becomes, the more the patient community, press and public are open to hearing and acting on the message.”

If invited, many companies can also present research data at patient advocacy organizations’ meetings and collaborate on articles that organizations may publish in their newsletters and share with members. Sharing trial results – and showing that scientific progress is being made on treating the disease – can help nonprofit organizations fundraise and build their capacity.

## Benefits to patient advocacy organizations

- Support to expand and develop the organization
- Connecting with the most current research; access to data
- Obtaining information and articles upon request
- Helping the patient community understand drug development and the clinical trial process

Companies can also help nonprofit organizations educate their members by sharing what to expect from clinical trials. “There will always be people who do not understand the difference between a Phase 1, a Phase 2, and a Phase 3 study,” said Gershkowitz. “And they’ll still look towards us as

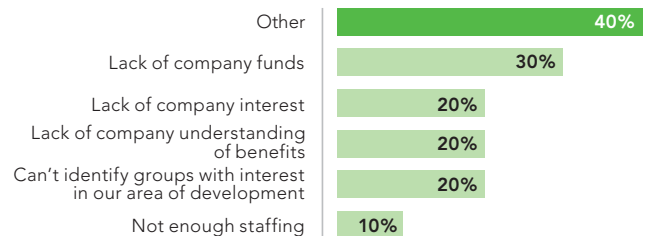
patient advocates – disease or company agnostic – and say, ‘Could you help us understand the whole process?’”

## Challenges for establishing a successful patient advocacy function

Industry patient advocacy functions can identify and possibly create programs mutually beneficial to companies, organizations and patients. However there are challenges to establishing a successful patient advocacy function.

As the BioNJ survey revealed, the number one barrier companies face in getting involved with patient advocacy is the lack of an adequate budget. Fully 30% of respondents cited budgetary constraints as the reason their company was not yet involved in patient advocacy, while only 20% cited a lack of company interest, and 20% a perceived lack of benefits. This makes sense, as 29% of respondents had fewer than 100 employees, 16% had zero revenue, and 32% had no products in the market.

## You indicated that your company is not involved in patient advocacy; please check all reasons that apply



Some of the challenges revealed by the survey and webinar experts include:

- An institutional discomfort within biopharmaceutical companies with interacting directly with patients. While meeting patients in-person can be inspiring to some, others may prefer not to meet patients.
- The rules and guidelines against soliciting nonprofit patient advocacy groups for involvement.
- “Because compliance issues are changing,” Gershkowitz said, “we make sure that we have written requests on file for updates from patient organization leaders and individuals interested in our programs, so that we are not perceived as proactively soliciting. This is a best practice we have developed.”



- Different global regulations and standards for working with nonprofit groups. This is particularly challenging in Europe, where the standards for engagement are much more stringent.
  - “Europe is [...] somewhat behind us,” said Goetz, “but they’re [...] catching on to the U.S. idea of what ‘patient power’ really means.”
- Communicating the internal value proposition of patient advocacy within the company.
- The availability of adequate budgetary resources and staff to support the patient advocacy function.

Many of these challenges can be overcome, especially by companies committed to reaping the benefits of talking – and listening – to patients.

## Best practices for patient advocacy functions

As Thompson said, “Patient advocacy is done best at a local level to meet local patient needs. It’s a time-consuming process, and that’s really the beauty of it and how we learn so much from it.”

Because patient advocacy is a relatively new strategic function in the biopharmaceutical industry, there’s much to gain from practitioners sharing ideas and best practices. Some of the advice shared by the webinar presenters included:

- **It’s not PR or marketing.** Patient advocacy functions are best at creating a free flow of information between patients and companies; they’re not for boosting sales.
- **Align objectives within the company.** Practitioners need buy-in from their colleagues across the company that patient advocacy is valuable to the company’s development pipeline, clinical success, and the reception of the product by the patient community. Thompson then explained, companies need to develop measurable benchmarks for the effectiveness of the function. “We do make a strong effort to bring our senior leaders to the table to sit down and hear from the patient advocates,” she said.
- **Engage with the community.** Patient advocacy requires significant travel and attending many of the advocacy organizations’ meetings and conferences. Visibility is key.

- **Make equitable funding decisions.** For some diseases and disorders, there are only one or two nonprofit advocacy organizations; for other disorders, there may be a dozen. “You don’t play favorites,” said Gershkowitz. “You don’t pick one over another.”
- **Treat patients with respect.** “Patients and advocacy groups can become important allies, and they must be treated with respect, honesty and transparency,” said Goetz.

Patient advocacy is still an emerging discipline and this paper seeks merely to introduce what a corporate patient advocacy function may entail, including the benefits and challenges. As the field matures, BioNJ – with the support of Merrill DataSite – will help to educate the community regarding patient advocacy and its important role in assisting the industry. This strategic function, and its practitioners, are becoming a significant driver in the growth and health of the life science industry in New Jersey and across the country. BioNJ is dedicated to providing information resources and facilitating networking and knowledge sharing for these practitioners within companies.

## For more information

View the [BioNJ Patient Advocacy Webinar](#) online. And check back at [www.BioNJ.org](http://www.BioNJ.org) for more resources on patient advocacy.



## About BioNJ

With more than 300 member companies, BioNJ is singularly focused on the growth and prosperity of New Jersey's life science industry. Founded in 1994 by New Jersey industry CEOs, BioNJ serves as the voice of biotechnology companies located in New Jersey, seeks to advance their economic growth and development and works to encourage new and established companies from around the world to locate in New Jersey. BioNJ represents companies engaged in biopharmaceutical, biomedical, bioagricultural and bioremedial endeavors.

As part of its mission, BioNJ is actively involved through its Patient Advocacy Committee in establishing a sustainable initiative to help facilitate the role of in-house Patient Advocacy functions for its Member companies. For more information please review the Web site or contact BioNJ at 609-890-3185, or [BioNJ@bionj.org](mailto:BioNJ@bionj.org).

## About Merrill DataSite®

Merrill DataSite ([www.datasite.com](http://www.datasite.com)) is a secure virtual data room (VDR) solution that optimizes the due diligence process by providing a highly efficient and secure method for sharing key business information between multiple parties. Merrill DataSite provides unlimited access for users worldwide, as well as real-time activity reports, site-wide search at the document level, enhanced communications through the Q&A feature and superior project management service - all of which help reduce transaction time and expense. Merrill DataSite's multilingual support staff is available from anywhere in the world, 24/7, and can have your VDR up and running with thousands of pages loaded within 24 hours or less.

With its deep roots in transaction and compliance services, Merrill Corporation has a cultural, organization-wide discipline in the management and processing of confidential content. Merrill DataSite is the first VDR provider to understand customer and industry needs by earning an ISO/IEC 27001:2005 certificate of registration – the highest standard for information security. Merrill DataSite is certified for operations in the U.S., Europe and Asia. Merrill DataSite's ISO certification is available for review at: [www.datasite.com/security.htm](http://www.datasite.com/security.htm). Learn more by visiting [www.datasite.com/lifesciences](http://www.datasite.com/lifesciences) today.

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Founded in 1968 and headquartered in St. Paul, Minn., Merrill Corporation ([www.merrillcorp.com](http://www.merrillcorp.com)) is a leading provider of outsourced solutions for complex business communication and information management. Merrill's services include document and data management, litigation support, language translation services, branded communications programs, fulfillment, imaging and printing. Merrill's target markets include the legal, financial services, insurance, healthcare and real estate industries. With more than 5,000 people in over 40 domestic and 22 international locations, Merrill empowers the communications of the world's leading companies.



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