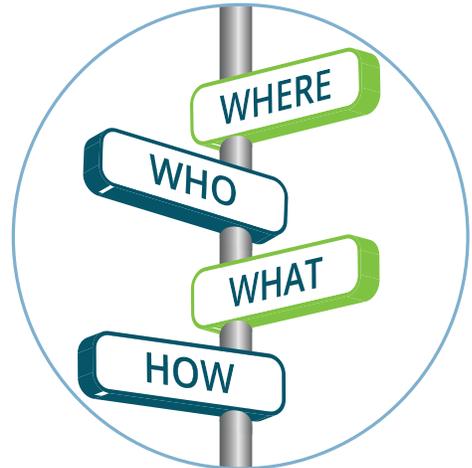




SEARCHING FOR ANSWERS: CONTACTING BIOPHARMACEUTICAL COMPANIES EFFECTIVELY

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Introduction

Undiagnosed and rare disease patients often face endless healthcare questions with few to no answers. Doctors frequently know little to nothing about their patients' rare disease, and with little treatment options available (if any), patients are often the ones taking the lead in driving and managing their own diagnosis and treatment.

Proactive patients and family members reach out to pharmaceutical and biotechnology companies on many important issues, including:

- The status of research
- Gaining access to investigational or approved medications
- Help with patient and prescription assistance programs
- Financial assistance to offset the burden of disease
- Reward program services
- Disease education
- Product information
- The drug development process
- Fundraising, grant, and sponsorship opportunities

Alison Rockett Frase, the Founder and Vice President of the Joshua Frase Foundation, says that as someone working to propel research for almost two decades, a patient advocate, and a mother of an affected child, she sees the importance of improving communication between rare disease patients and their families with biopharmaceutical companies.

“Increasing awareness and education for underrepresented populations will ensure a more open communication between all, and a more equal and balanced enrollment of the population in a clinical trial,” Alison Rockett Frase says. “This must be a team effort for all parties involved in order for success to be achieved on all levels.”

This toolkit breaks down the process of reaching out to the biotech and pharmaceutical industry effectively and the steps you can take to get answers.

SECTION 1:

PREPATORY WORK



Before contacting biopharmaceutical companies, it is important that patients do their homework. Beyond a simple Google search for names and addresses of companies, there is additional information to collect that will help patients communicate more effectively with a company. Some questions they should be prepared to answer include:

- What is the company's mission and vision?
- Which disease(s) is its primary focus?
- Who are the company's leaders (such as its executive team, board of directors, scientific advisory board)?
- What does its drug development pipeline look like? Are there any approved drugs?
- What is its structure (departments, locations, etc.)? Does the company list resources for patients and physicians?

Much of this can be found on the company's website in its **About Us** section or under related links. In addition to Google and Bing searches, if you still need help finding a company, check this online directory at **World PharmaNews** (<http://www.worldpharmanews.com/directory>). If a company is public, filings such as registration statements or annual reports filed with the **U.S. Securities and Exchange Commission** can provide a wealth of information (<http://edgar.sec.gov/edgar.shtml>).



Insider Tip:

"Before reaching out to companies (large or small), it would be helpful to be educated on the status of research on the disease," says Andra Stratton, Co-Founder of Lipodystrophy United, an organization that has worked with the pharmaceutical and drug companies associated with its disease.

A few additional questions to investigate, according to Andra Stratton, include:

- Are there clinical trials (www.clinicaltrials.gov) in any phase?
- Is there a mouse model (try googling it)?
- Are there any treatments and/or medications being used regularly, if no disease specific treatment is available?

Since the biopharmaceutical industry may develop drugs for various rare diseases, it would be helpful to see what other patient organizations may be of interest to them. In addition to researching the company, it is important to research other resources. There may be patient advocacy organizations or online disease communities that can provide guidance and support.

If There is a Patient Organization

Recently, biopharmaceutical companies have recognized the importance of engaging with patient advocacy organizations when planning their drug development strategies. By connecting with organizations, the likelihood of establishing and maintaining an ethical and responsible relationship increases. It is important for the company to hear patients' voices and to understand the community's needs.

Companies will be more open to establishing strong relationships with patient organizations than individual patients. Issues that are raised by an organization to a company usually get directed to the right person faster. Before becoming the Director of Global Research of Development at the International FOP Association, Betsy Bogard worked at Genzyme, Genentech, and VaxGen. She offers some perspective from the other side of why companies engage more with patient organizations than single patients and their families.

"Companies do and should have direct engagement with patients—it's important,

SECTION 1: PREPATORY WORK

helpful, and productive. However, I believe in many cases these engagements are best done with the awareness or involvement of the relevant patient organization, if one exists. The patient organization typically has context and insight into the wider disease community. They may have access to experts and professional advisers who can inform the dialogue. They can help ensure fairness and transparency within the community.

“Given their responsibility for representing and organizing the community, they should have the opportunity to learn from any disease insights that a patient may share with a pharmaceutical company, or corporate insights the company may share with the patient or family. Suppose there is a conversation between a pharmaceutical company and one patient or family. Is the information the patient shares representative of the majority of the community, or more of an interesting and informative exception to the norm? A patient organization can help shed light on that.”

If you are an individual wishing to be involved with a patient organization, here are few suggestions when doing homework on them:

- Visit the patient organization page to gather information on its leadership (staff, board, and medical advisors)
- Spend time on webpages to become familiar with their programs, how they are funded, and if they support research
- Volunteer to help with an event, attend a conference— get to know the organization before elevating your level of commitment.
- If further details are needed, visit **GuideStar** (www.guidestar.org) for financial information on nonprofits registered with the IRS
- Additionally, if more information is needed, reach out to its leadership (the executive director, board president, or board member)

and learn which companies may be working in the disease space

- Inquire if any others are involved with or exploring the disease area, including academic researchers
- Ask about the organization's ongoing communications/relationship process with companies and how you can receive updates

If you are interested in finding and joining a patient advocacy organization, the National Center for Advancing Translational Sciences Office of Rare Diseases Research has compiled a **list of advocacy organizations** that support rare diseases (http://rarediseases.info.nih.gov/files/Patient_Support_Groups_ORDR%20List.pdf).

If There is NO Patient Organization

If no patient organizations exist for a patient's disease or disease group, the approach will be similar. It is still recommended that patients try to find others who can support them. Some recommended steps if there are no nonprofit patient organizations available include:

- Utilize social media: join Facebook groups, listen to others to get a feel for the group's tone, share and post inquiries, collate information on the status of research, reach out to others who have successfully contacted companies, and learn from their experiences (A list of social media sites are listed in the **Resource Guide**)
- If there is a loose group, meet and decide how to proceed; gauge the interest to move forward with new ideas
- Reach out to academic researchers through existing academic scientific/medical contacts and the **National Institutes of Health Office of Rare Disease Research** (<http://rarediseases.info.nih.gov/>)
- Contact other patients and affiliated rare disease organizations that have successfully worked with biopharmaceutical companies in the past and ask for their advice

SECTION 2: CONTACTING THE COMPANIES



“Two potential deterrents for reaching out to companies are intimidation and fear of the stigma of big pharma,” says Andra Stratton, Co-Founder and President of Lipodystrophy United. “While identifying the correct person to speak with may be more difficult at larger companies, my experience has been that there is always someone who sees the value of partnering with the patient in some way. The patient is the true expert and your job is to let them know that it is only to their benefit to work with you.”

Many biopharmaceutical companies understand the importance of connecting regularly with advocacy organizations and patients for the disease areas they serve. Patients provide insight, feedback, and sometimes data relative to companies’ programs and products.

Companies involved with patient communities may also be able to see educational opportunities that could help affected individuals and/or their healthcare providers. It’s important to note that not all companies—especially startups and smaller ones—have established activities or are engaged with their respective disease or patient community. It’s important for patients to realize they may be one of a company’s first interactions with the community.

It is important when contacting companies to develop a strategy. Patients should ask themselves:

- What are my objectives for contacting them?
- Do I have a message prepared?
- Do I know who I should be reaching out to?

Formulating an Objective

It may be obvious to some, but it’s crucial that patients know what they want to accomplish before contacting a company. Here are some examples:

- Obtain financial support for a local 5K run to reduce a family’s medical expenses
- Secure lead sponsorship for an organization’s annual fundraising gala
- Get introduced to the lead researcher working on their disease to help them connect with the patient experience of diagnosis and disease management
- Obtain early access to an unapproved drug currently in clinical trials
- Request an article be submitted about the company and its work for an organization’s newsletter
- Inquire about diagnostic testing for their disease

Keep in mind that the first time a patient approaches a company, it might be better to offer something rather than ask for something. An example of what patients can bring to the table is included in the sample message below, which is both a request and an offer. The color coding helps illustrate this give and take, which is what a potential partnership should be built upon. Any company, be it long-established or startup, will appreciate this thinking from someone seeking a connection.

Also, patients should know if they will be representing the disease as an individual or family member, part of a group with other patients, or as a member or leader of a patient organization. If the main objective is to spark interest in your disease or research being done for it, turn to the **Appendix**. Betsy Bogard of the International FOP Association has provided some things to consider before making this connection.

SECTION 2: CONTACTING THE COMPANIES

Preparing a Message

When approaching biopharmaceutical companies, a well-crafted message can set a rare disease patient apart. They should identify themselves and their disease, but avoid providing an extensive description of the diagnostic journey or medical history in the initial email, voicemail, or call. The person on the receiving end may not be comfortable with this level of personal disclosure, and because of the lack of familiarity with patient interactions, or even company guidelines for such interactions, this discomfort may deter them from helping. And please don't expect medical advice from an industry representative.

Here is an example from Jayne Gershkowitz, the vice president of patient and professional advocacy and public policy at Amicus Therapeutics, of a well-crafted initial message (Note that the **blue** text shows the request/reason for contact, while the **green** text shows the offer or what a patient could bring to the table):

"I am Jane Doe, mother of John Doe, a 3-year-old recently diagnosed with _____ Syndrome (link to description on <http://rarediseases.info.nih.gov/> or <http://www.omim.org/> if it exists). I am a volunteer collaborating with other families affected by _____ Syndrome and seeking information about potential treatment. I am contacting ABC Bio to **learn more about your program for _____ Disease** because of the strong similarities this disease seems to have with _____. **Our families are very interested in any scientific or clinical research you are doing and to share with you our disease experiences, or any other information that may be helpful to your company.** Please let me know a convenient time for us to talk. Thank you very much."

Finding Who to Contact

Biopharmaceutical companies receive a lot of inquiries from patients and their families. Therefore, knowing the proper person to contact ensures that your message gets

read and answered, and the next steps get set into motion.

Finding the appropriate person to contact at a company can usually be done easily through online searches. Look at the company's organizational structure on the company's website. Is there a patient advocacy, medical affairs, public affairs, public relations, community relations, or corporate communications department? If not, are there descriptions of positions in the company that reflects people with direct interaction with the rare disease community the company serves?

If information is not easily available, call and ask the receptionist if there is a person who interacts with the patient community. For small companies, employees often wear multiple hats and there could be one or several people who perform this function with different, often vague titles.

One of the last options is calling and asking for the contact information for the head of Business Operations. These people may be able to help you, but typically don't have direct interactions with the patient community. Contacting the CEOs should always be seen as a last resort, and only should be considered by people who have a direct connection with them.

All of the above is based on reaching out to a company's headquarters. If a company of interest has a regional or field office, patients may consider reaching out to geographically based staff, such as field-based genetic counselors, case managers, or other regionally assigned patient services specialists.

Note:

Avoid contacting clinical research or clinical operations. Industry guidelines almost always forbid these departments from interacting directly with the patient community.



SECTION 3:

REQUESTING FINANCIAL SUPPORT



Financial support is a common request many companies receive, so knowing the appropriate person to reach out to is a good first step. Begin by searching the company's website for any company contributions, citizenship, or giving program information. Make sure to print and read the company's

- Giving guidelines
- Priorities
- Application process
- Forms to download
- Online process

If there is no information available, then contact patient advocacy, public relations, corporate communications, or medical affairs.

Patients should know what they want before reaching out to a company, such as event sponsorship, general operations support, educational grant, or corporate membership. They should ask about the company's giving program and its guidelines and provide a brief description of their request. Most companies will not be able to make grants to individuals, but only to 501c3 nonprofit organizations.

Also, due to the **Physician Payment Sunshine Act** imposing greater scrutiny of grants within the industry, companies cannot make arbitrary decisions on which organizations and activities to support. To learn more about the Sunshine Act, the **American Academy of Orthopedic Surgeons** (<http://www.aaos.org/news/aaosnow/mar13/cover3.asp>) provides ample information.

If patients have a connection at the company who knows their organization or disease, they should let them know ahead of time that they will be contacting the company and mentioning them.

RESOURCE GUIDE



Connecting with Patients and Medical Researchers

Facebook Groups (<http://www.facebook.com/help/>): Facebook reaches more than 1 billion monthly active users, making it easy for users to find and connect to others through supportive community groups.

Google Groups (<https://groups.google.com/forum/?fromgroups#!overview>): Google Groups lets users participate in online discussions. This social media site allows users to stay organized.

Inspire (<http://www.inspire.com>): Inspire connects patients, families, friends, caregivers and health professionals, providing health and wellness support.

The NCATS Office of Rare Diseases Research (http://rarediseases.info.nih.gov/files/Patient_Support_Groups_ORDR%20List.pdf): This link goes directly to a list of advocacy organizations that support the rare disease community. Before reaching out to a biopharmaceutical company, patients should connect with an advocacy group.

NIH Office of Rare Disease Research (<http://rarediseases.info.nih.gov/>): The Office of Rare Disease Research coordinates and supports rare diseases research, responds to research opportunities for rare diseases, and provides information on rare diseases. Contact information on academic researchers can be obtained through this office.

PatientsLikeMe (<http://www.patientslikeme.com/>): Users can join this registry to connect with others in similar situations. Access this site to read through other patients' stories, learn from their experiences, and share your own.

Rare Connect (<https://www.rareconnect.org/en>): Hosted by trusted patient advocates, this is a place where rare disease patients can connect with others globally. Joining or forming a group through Rare Connect can help users provide support to others, while learning from their experiences.

Rare Share (<http://www.rareshare.org/>): RareShare is a social hub that helps build communities for patients, families, and healthcare professionals affected by rare conditions.

Yahoo! Groups (<http://groups.yahoo.com/>): With a long list of groups, it's easy to find an appropriate one through this site. Establishing a group through Yahoo! allows users to connect with a world of rare disease patients and parent advocates, inspiring and empowering others.

RESOURCE GUIDE



Connecting with Rare Disease Umbrella Organizations

Canadian Organization for Rare Disorders (<http://raredisorders.ca/>): CORD is Canada's national network for organizations representing all those with rare disorders. CORD works with governments, researchers, clinicians, and industry to promote research, diagnosis, treatment, and services for all rare disorders in Canada.

EURORDIS (<http://www.eurordis.org/>): EURORDIS is a non-governmental patient-driven alliance of patient organizations and individuals active in the field of rare diseases in Europe.

Global Genes (<http://globalgenes.org/>): This nonprofit organization strives to eliminate the challenges of rare disease by developing educational resources, providing connections, and equipping patients to become successful activists.

Japan Patients Association (<http://www.nanbyo.jp/>): The JPA is a nonprofit umbrella organization established in 2005. It focuses on rare and intractable diseases and has a membership of approximately 300,000 from 72 organizations, including individual patient groups and regional centers. JPA provides patient/family services and advocacy on public policies. It also collaborates with other organizations in Japan and organizes patient forums.

National Organization for Rare Disorders (<https://www.rarediseases.org/>): NORD provides information for patients and families, mentoring for patient organizations, advocacy for the rare disease community, research grants, and patient assistance programs.

New Zealand Organization for Rare Disorders (<http://www.nzord.org.nz/>): NZORD helps people affected by rare disorders and their families to find essential information; provides resources and information for rare disease support groups; monitors rare disease issues and policy matters; and builds partnerships between patients/families, support groups, clinicians, researchers, policy-makers and industry.

Rare Voices Australia (<https://www.rarevoices.org.au/>): The unified voice for all Australians living with a rare disease, Rare Voices Australia provides a strong common voice to promote for health policies and a healthcare system that works for those with rare diseases.

Taiwan Foundation for Rare Disorders (<http://www.tfrd.org.tw/>): The Taiwan Foundation for Rare Disorders was founded to provide support for rare disorder patients, who are the minority among minorities of Taiwan society. This organization believes that this community's quality of life will be improved through encouraging medical research and establishing formal procedures in the referral, diagnosis, treatment, and care of rare disorder patients.

RESOURCE GUIDE



Learning More about a Company

Clinicaltrials.gov (<http://clinicaltrials.gov>): This website provides a listing of all current clinical trials being done in the United States and around the world. Use the search box to find them easily.

CheckOrphan (<http://checkorphan.org/>): A nonprofit organization dedicated to rare, orphan and neglected diseases, CheckOrphan allows users to be updated daily on all the latest news. Subscribe to their news to receive regular email updates.

FasterCures (<http://www.fastercures.org>): A center of the Milken Institute, this action tank is driven by a singular goal: to save lives by speeding up and improving the medical research system. Subscribe to their news and read updates on biopharmaceutical companies, their products, and their programs by clicking “Connect” on the FasterCures homepage.

GuideStar (<http://www.guidestar.org/>): This information service gathers and disseminates information about every IRS-registered nonprofit organization. It provides information on each nonprofit’s mission, legitimacy, impact, reputation, finances, programs, transparency, governance, and more.

Orphan Druganaut Blog (<http://orphandruganaut.wordpress.com/>): This resource provides competitive intelligence, news, articles, and internet buzz on global orphan drug developments and rare diseases. Turn to this resource for updates on products in the drug development pipeline, analytic overviews of pharmaceutical marketplace, and identifications of domestic and international Key Opinion Leaders.

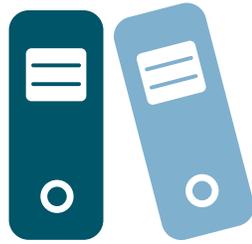
Rare Disease Report (<http://www.raredr.com/>): This website and weekly e-newsletter offers a voice for the rare disease community. It strives to bring together medical, scientific, investment, regulatory, and advocate professionals interested in rare diseases and orphan drugs. Subscribe to its newsletter to learn more about biotech and pharmaceutical companies, their endeavors, and their recent drug approvals.

WorldPharmaNews (<http://www.worldpharmanews.com/directory>): This online directory makes it easy to find the contact information of biotech and pharmaceutical companies.

Understanding the Sunshine Act

American Academy of Orthopedic Surgeons (<http://www.aaos.org/news/aaosnow/mar13/cover3.asp>): The Physician Payment Sunshine Act allows for increased transparency about the financial relationships between physicians and industry. The Academy published articles, including this one, on its website to provide a broad overview of the Sunshine Act and its regulations.

APPENDIX:



ENGAGING PHARMACEUTICAL COMPANIES

Betsy Bogard, International FOP Association

When you want to engage a pharmaceutical company to generate interest in your disease, here are a few thoughts that might help:

Be Selective: Pick companies that might have a reason to be interested in the disease, first and foremost, because they have a technology that is plausible for it, and because the disease fits with the company's strategic interests. Many companies today are interested in even the rarest of diseases if there is potential that their technology could help address it.

Know the Company: Read anything you can about the company so that you know how the company evolved, what news they have reported recently, who leads the company and what their background is, and what constraints or issues the company may be dealing with.

Provide a Disease Overview: Prepare a short overview, perhaps 5-7 slides long, that summarizes:

- The cause (if it is genetic, the known defective genes)
- The course (what is the age of onset, first symptoms, typical progression, etc.)
- The impact (its severity, its life span)
- The epidemiology (its prevalence; its racial, ethnic, or geographic predispositions)
- The state of basic and preclinical research on the disease (e.g. are there animal models available?)
- The treatment landscape (any therapies already available, potential therapies in development, plausible therapeutic approaches, etc.)
- Any medical and patient networks around the disease (established medical networks, research consortia, patient organizations, databases, or registries)

This is much of what a company considers when they decide whether to pursue a disease.

Connect them to disease experts: There is only so far that patients and families can go in exploring possible technologies. At some point, if they have any interest, the company needs to talk to academic and medical experts. Offer to make that connection to help them get answers to technical questions, or even to explore a pilot project.

Follow-up: *Always* follow up to say thanks for any time and attention the company has given. You can send more detailed information, such as a couple of relevant publications, afterwards. If the company shows interest but isn't getting back to you, follow up again—everyone is busy, and they may just need a nudge. But if nudging doesn't go far, move on to another company. Ultimately, you want a company that is as interested in you as you are in them.



**LET OTHERS BENEFIT
FROM YOUR KNOWLEDGE
BY SHARING YOUR TIPS
AND TRICKS!**

If you would like to contribute your experience
or have a comment/suggestion,
please enter it online at <http://globalgenes.org/toolkits>.





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