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Crowdfunding for Personalized Medicine Research

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Given the current funding situation of the National Institutes of Health, getting funding for rare disease research is extremely difficult. In light of the enormous potential for research in the rare diseases and the scarcity of research funding, we provide a case study of a novel successful crowdfunding approach at a non-profit organization called Rare Genomics Institute. We partner with biotechnology companies willing to donate their products, such as mouse models, gene editing software, and sequencing services, for which researchers can apply. First, we find that personal stories can be powerful tools to seek funding from sympathetic donors who do not have the same rational considerations of impact and profit. Second, for foundations facing funding restrictions, company donations can be a valuable tool in addition to crowdfunding. Third, rare disease research is particularly rewarding for scientists as they proceed to be pioneers in the field during their academic careers. Overall, by connecting donors, foundations, researchers, and patients, crowdfunding has become a powerful alternative funding mechanism for personalized medicine.

Three hundred million people worldwide suffer from rare diseases [1], but treatments are available for less than 5 percent of those 7,000 known diseases. Because most of these diseases affect very few individuals, getting funding for research from traditional sources is extremely difficult, especially under current conditions [2]. In light of the enormous potential for research in rare diseases and the scarcity of funding, we would like to provide a case study of a novel crowdfunding approach that we found to be successful at a non-profit organization called Rare Genomics Institute (RGI†).

For people with undiagnosed rare diseases who may have been to dozens of doctors with no clear answers, genome and exome sequencing offers a new way to discover the root causes of diseases and, in some cases, a diagnosis of a known disease. Through an online crowdfunding platform and network of sequencing sites, RGI trains families of children with undiagnosed rare diseases how to run an effective crowdfunding campaign, as insurance often fails to cover the costs of sequencing [3]. Top genetic scientists who share a passion for helping rare disease patients provide pro-bono assistance in interpreting sequencing results, researching likely diseases, and identifying the most promising specialists and clinical trials for patients.

However, even if a diagnosis is secured through sequencing, the sad truth is that cures and effective treatments are seldom available. Frequently, this is not due to the inherent difficulties in developing a successful therapy for any one rare disease, but the severe lack of both private and public sector funding to conduct research and clinical trials for diseases that affect a relatively small number of people. To help resolve this, we partnered with companies to establish an effort for rare disease research called BeHEARD (Helping Empower & Accelerate Research Discoveries).

HOW IT WORKS

Our organization partners with biotechnology companies, primarily those in the rare disease field, that are willing to provide in-kind donations of their products,

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†Abbreviations: RGI, Rare Genomics Institute; BeHEARD, Helping Empower & Accelerate Research Discoveries; NIH, National Institutes of Health.

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such as mouse models, gene editing software, and sequencing. Through BeHEARD, we offer these products as free technology grants to researchers studying underfunded rare diseases. Foundations and academic institutions apply for specific technologies, filling out a grant application that explains how the technologies would be used to further research on a specific rare disease. Applications for each technology are evaluated by a panel composed of a representative of the company that supplied the given technology and scientific experts in the rare disease field. Each winner of a specific technology is then entered into a grand prize competition for a cash research grant, the winner of which is determined by a combination of social media voting and the opinions of expert panel.

WHY DO COMPANIES SPONSOR?

1. Donating free products as opposed to cash is a simpler and more cost-effective way for companies to support researchers. In general, it is easier for company representatives to get approval to donate products than money.

2. By providing a technology, companies gain exposure for their products with their target market, as potential applicants browse the technologies. If companies were to provide a cash grant instead, potential applicants might not bother to see what the company's products were.

3. Winners of a technology who find the product useful and have a good experience with the company may become long-term customers.

EXAMPLES OF PROGRESS: SANFILIPPO SYNDROME

Sanfilippo syndrome has benefitted the most from crowdfunding models of fundraising. Sanfilippo syndrome is a lysosomal storage disease, caused when lysosomes have problems breaking down unwanted materials and instead store them inside the cells. This causes apoptosis, including degeneration of the central nervous system. There is no cure or effective treatment, and children die after losing the ability to walk, talk, and eat.

Jill Wood was one of BeHEARD's science challenge winners last year. Jill and her husband founded Jonah's Just Begun, an organization created to find a cure for Sanfilippo syndrome Subtype D after their son, Jonah, was diagnosed with the rare disease. Jill Wood won a knockout mouse for Sanfilippo Subtype D from Taconic, which provided the groundwork for Jonah's Just Begun's scientists to better understand the disease and propose research for future treatments. As a result, the foundation won a National Institutes of Health (NIH) grant for $223,102 for the first pre-clinical research for a treatment for Sanfilippo Subtype D.

Another example is Saving Eliza, a crowdfunding campaign set up by the parents of a child diagnosed with Sanfilippo syndrome Subtype A after they discovered that researchers had found a potential breakthrough gene therapy for the untreatable disease. However, the researchers had been unable to act on it, because due to the disease's rarity, it was nearly impossible to find funding for clinical trials. To date, Saving Eliza has been one of the most successful crowdfunding campaigns in history, with nearly $2 million raised.

LESSONS FOR RESEARCHERS, FOUNDATIONS AND INDIVIDUALS WITH RARE DISEASES

We propose that crowdfunding, with an emphasis on individual stories, may be a more effective method of raising money for rare disease research than traditional funding from organizations such as the NIH. Governments, with an obligation to spend their money to help the most people, naturally will gravitate toward prevalent diseases, even if it may be easier to develop treatments for rare ones. Similarly, biotechnology companies, seeking to maximize profits, often will not see research for diseases with very few sufferers as a good investment. For rare diseases, personal stories can be powerful tools to seek funding from sympathetic donors who do not have the same rational considerations of impact and profit.

For foundations and researchers facing funding restrictions, company donations can be a valuable tool in addition to crowdfunding. However, instead of requesting financial assistance, based on our experience with BeHEARD, we believe organizations will have more success approaching companies with a request for in-kind donations to support their research.

Finally, for scientists who wish to make a difference with their research, studying rare diseases may seem to be less attractive than focusing on widespread diseases that affect large numbers of people. However, rare disease research is particularly rewarding for scientists who choose to make it their field of study. As we see with Sanfilippo syndrome, instead of an incremental increase in knowledge on high profile, difficult-to-treat diseases such as cancer, there is true potential for individual researchers, labs, and foundations to find treatments and cures for diseases that have not previously been researched at all.

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