The Pulse on Global Trials

By Matthew Howes

Earlier this year, the FDA approved the first artificial pancreas for type 1 diabetes. The advancement is remarkable not just for the technology alone, but for how it came to be.

The Juvenile Diabetes Research Foundation (JDRF) spent more than $100 million to fund research for the new device, which automates the process of regulating blood sugar in patients with type 1 diabetes. Over the last decade, JDRF recruited academic researchers, global companies and federal legislators to advocate for the breakthrough medical device through regulatory approval.

This marks a significant expansion of the role patient advocacy plays in drug development. Around the world, biopharma companies are working with patient advocacy foundations earlier, more frequently and more closely than they have in the past. As the relationship between advocacy and industry evolves, insights are being uncovered upstream in the therapeutic discovery process, which allows scientific advances to gain regulatory approval faster and more efficiently.

In recent years, a number of critics have voiced concern that Patient Advocacy Groups (PAGs) are simply another channel for the industry to push its agenda. The traditional advocacy model is characterized by a flow of money from biopharma companies to PAGs, who use the funds to educate patients and advocate on their behalf to influence policies that support their interests. This money, it is argued, makes PAGs less of an independent voice than they should be.

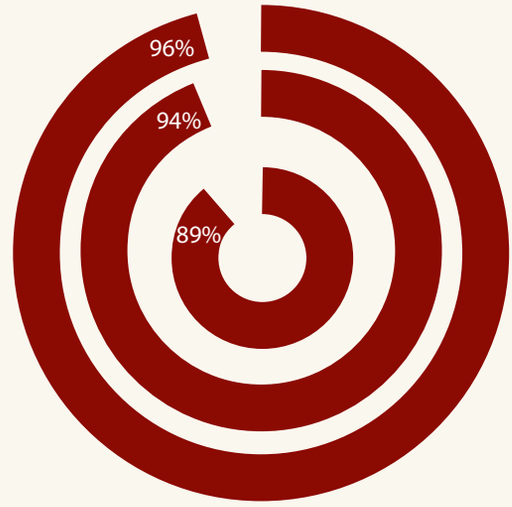
However, new models for the role of PAGs and how they are funded are emerging in a number of regions around the globe. In the EU, for instance, advocacy groups receive government funding for their education and policy initiatives to raise awareness of the need for innovative drugs and devices for rare diseases, cancer and other conditions. PAGs in the EU sit on advisory boards and committees of the EMA, ensuring that the patient voice is heard by all stakeholders.

Patient advocacy in clinical research

96% of PAG's get their information about the RDCRN from the patient community

94% of consortium principal investigators' are involved in patient recruitment

89% of PAG's are involved in patient recruitment



In Latin America, the shift in the disease burden from infectious diseases to chronic conditions is a driving factor for the increasing influence of patient advocacy. In Mexico and Brazil, for example, advocacy groups are growing in disease areas such as breast cancer and diabetes, and are starting networks supporting patients undergoing treatment for the respective diseases. As a result, an increasing number of patients and survivors are promoting change in the public health system to ensure better quality of care and access to treatments in Latin America.

In the U.S., the Rare Diseases Clinical Research Network (RDCRN), part of the NIH, has expanded its research efforts to include collaboration with representatives of 98 patient advocacy groups to advance clinical research and investigate new treatments for patients with rare diseases. Working together earlier in the process, they hope to bridge the gap between preclinical work and patients.

The RDCRN is designed to advance medical research on rare diseases by providing support for clinical studies and facilitating collaboration, study enrollment and data sharing. Through the RDCRN consortia, physician scientists and their multidisciplinary teams work together with PAGs to study more than 200 rare diseases at sites across the nation.

Beyond representing the voice of the patient in clinical research, PAGs are also stepping up to be an active watchdog in the broader healthcare ecosystem. PAGs have petitioned the NIH and FDA to better enforce reporting of results from human testing of new treatments. According to STATnews.com, many of the top research institutions regularly fail to comply with a federal mandate to publically report clinical trial outcomes.

The healthcare industry has been striving to be more patient-centric. The ability of foundations like JDRF to independently source and fund new research is changing the leverage PAGs have in drug development. It is also giving patients a more valuable voice in helping researchers comply with regulations that are meant to put patients first. PAGs' evolving role, and industry's willingness to collaborate with them, will continue to be important to achieving patient-centricity.

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