Worldwide Clinical Trials

Why the Evolution of Patient Advocacy Organizations Means Better Data for Your Rare Disease Trial

Rare disease drug development is a unique and challenging field that demands strategic development expertise and uniting of the various stakeholders working toward the same goal.

As a Therapeutic Strategy Lead for Rare Disease at Worldwide Clinical Trials, Juliane Mills is invested in seeing patient communities continue to evolve as influential players in rare disease research. Their transformation from advocacy organizations to research organizations holds great promise, empowering all stakeholders in rare disease research to find better treatments and cures.

"Rare disease communities have evolved and helped to mold the research landscape over the last 10 to 15 years. What I am seeing now, and it's so important, is a shift in the influence and role of patient-led research communities in rare disease trials. Many of these organizations have funded the basic research, assembled a contact registry, and created a network of investigators — all resources which are so valuable to drug development," Mills said.

The Evolution Into Patient-Led Research Organizations

Historically, rare disease communities were more focused on supporting their communities. Over time and through the support and trainings offered by many government and umbrella organizations, such as the National Organization for Rare Disorders (NORD) and International Rare Diseases Research Consortium (IRDiRC), these communities have been able to broaden their mission beyond patient support.

Patient communities now conduct their own disease research, and this empowerment is transforming the rare disease drug development landscape. These communities are increasingly equipped with preclinical models, cell lines, animal models and research funding, all of which are critical tools for a drug development sponsor.

They also conduct observational research that helps validate endpoints and measurement tools specific to their indications. According to Mills, these are welcome changes.

Juliane K. Mills

Therapeutic Strategy Lead, Rare Disease

Meet Juliane

"I love to see the growing empowerment of these communities and how sponsors have embraced their collaboration. Seeing patient communities own their data and come to the table with drug developers as equal stakeholders has been transformational for the pace and quality of rare disease research," Mills said.

Contributing to Better Endpoint Selection

This selection of endpoints is where Mills thinks sponsors and patient-led research organizations have a real opportunity to work together. At the end of the day, regulators (and investors) are looking for proof that a product is effective in a given population. The quicker a drug developer can secure that evidence, the faster it will receive funding to move on to the next stage of drug development.

"Rare disease patient communities may already have the data you want, like feedback on endpoints or treatment sentiments. The community may have already done that research on their own, potentially saving the sponsor time and money."

"The selection of endpoints can impact the ability of sponsors to secure funding for additional development and to attract major pharma partners in development," Mills emphasized.

However, the choice of endpoints is not a one-size-fits-all approach: "Unfortunately, some rare disease indications are progressive. If the endpoint you've selected is a measure of something like neurocognitive development or muscle strength, changes in those abilities can happen very slowly in some diseases. While slow progression is fortunate for the patient community, for the drug developer, using those endpoints can mean that it takes longer to measure meaningful change and may require running a two-year study instead of a six-month study," Mills explained.

Collaborating with patient communities can expedite development by tapping into existing data and research efforts.

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While the ultimate goal for patient-led research organizations is to cure or prevent rare disease, in the short term, patients and families need treatments for symptoms significantly impacting their quality of life. Treatments that mitigate endpoints like sleep, continence, or duration and severity of seizures are not cures for the underlying disease but would still be helpful for families dealing with rare diseases. Including endpoints in trials that measure these symptoms can be a stepwise approach to development.

"At Worldwide, we have the expertise and experience in rare disease research to help reduce risk to a drug trial timeline. One of the ways we do that is by assisting with endpoint selection by considering the questions patients want answered together with the data that regulators and investors expect. If a drug developer chooses the wrong endpoint, they may not be able to enroll their study or may not have the evidence to convince investors or regulators that their product is effective. That's why endpoint selection is so critical," Mills pointed out.

Supporting Your Partnerships With Patient-Led Research Organizations

Worldwide's rare disease team has a long history of working with patient communities and has partnerships with more than 100 patient-led organizations across the globe. They focus on building mutually beneficial relationships with these organizations to drive sustainable partnerships between industry and patients that will advance both clinical development and patient agendas. They excel at mapping out a strategy designed to support the research that patient communities want and to collect the data that sponsors need to advance product development.

While expertise and experience are crucial, Mills said, passion is the true differentiator for those working in rare disease drug development. That passion drives sincere partnerships rooted in a shared mission and sense of obligation.

Few people choose rare disease research — most are called to this work because they have a family member or loved one directly affected by a rare disease. This personal investment often creates an all-consuming determination to work towards treatments and cures. It is work that requires like-minded partners who understand the objective and share the sense of urgency.

"So much is on the line for the teams supporting these rare indications. They're up at 3 a.m., refreshing their dashboard to see what's happening with their study. Partnering with a CRO who understands, shares that investment, and will be up refreshing the dashboard alongside them, makes all the difference," Mills added.

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