CLINICAL TRIAL DESIGN

EMBEDDING THE PATIENT & CAREGIVER VOICE

A summary of Alexion's Listening & Learning Webinar on July 14, 2022

Given the small patient populations associated with each rare disease study, **the perspectives of every patient and caregiver are essential** to designing a meaningful clinical trial and increasing the likelihood of its success.

» Orphan drugs or biological products, granted this designation by the FDA, have the potential to prevent, diagnose or treat a rare disease or condition.

» An orphan disease is a rare disease or condition that affects **fewer than 200,000** people in the United States.[2]

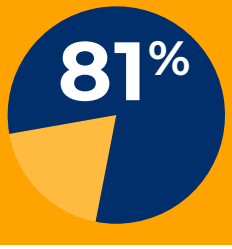
CLINICAL TRIALS OF ORPHAN DRUGS HAVE ABOUT

1/3

AS MANY PARTICIPANTS

AS TRIALS INVOLVING NON-ORPHAN TREATMENTS.[1]

OF PATIENTS SCREENED FOR RARE DISEASE CLINICAL TRIALS



81%

DO NOT MATCH THE ENROLLMENT CRITERIA.[3]

Meeting eligibility requirements for rare disease clinical trials is often more difficult than for non-rare diseases – contributing to an already limited pool of trial options. Because entry can be a challenge and is sometimes as rare as the disease itself, every trial opportunity counts.

"It is really important to understand the unique burdens of each patient community."

"At the end of this very long, very scientific, very research heavy drug development process is someone who is going to use this life saving medication, so we need to get input from them all the way through the process."

ACROSS NEARLY

30,000

clinical trials studying

1,535

rare diseases

70%

were studied in less than

10

trials and

28%

were studied in only

1

trial.[4]

EVERY TRIAL OPPORTUNITY COUNTS

ONE-QUARTER OF RARE DISEASE TRIALS WERE TERMINATED between 2016 and 2020 due to low accrual rates.[5]

Direct insights from patients and caregivers ensures that a trial reflects the real-world experiences of the people affected by a disease. By incorporating their voices early and often, clinical trial protocols can be shaped with the community in mind. This allows for greater access to trials, eases long-term participation and promotes more impactful trial results.

HALF

OF ALL RARE DISEASES AFFECT INFANTS AND CHILDREN.[6]

For these patients, caregivers are vital to participation in a clinical study. It is important to consider the burden frequent travel, hospital stays, and other requirements of a trial may have on patients as well as their support system, which can include parents and siblings, among others.

"This is personal. This is our lives. At the end of the day, this is about people."

"With a 'typical disease,' very often the patient is self-sufficient. In rare disease, it becomes very often a family business. You need a whole setup around a patient to be able to attend a clinical study."

CLINICAL TRIAL RESOURCES

- » ClinicalTrials.gov
- » Orpha.net
- » NHGRI

"When you are treating a patient, when you are treating a person, you are treating a family and an extended family."

ADDITIONAL RESOURCES

- » NORD
- » GARD
- » Global Genes
- » One Rare

FOR U.S. AUDIENCES ONLY

1. Institute for Clinical and Economic Review. The Next Generation of Rare Disease Drug Policy: Ensuring Both Innovation and Affordability. ICER-White-Paper_The-Next-Generation-of-Rare-Disease-Drug-Policy_040722.pdf. Accessed July 2022. 2. FDA.gov. Orphan Products: Hope for People With Rare Diseases. https://www.fda.gov/drugs/information-consumers-and-patients-drugs/orphan-products-hope-people-rare-diseases. Accessed July 2022. 3. Getz, K. Proliferation of Rare Disease R&D Necessitating Novel Strategies. Applied Clinical Trials. Volume 28 Issue 9. Available at https://www.appliedclinicaltrialsonline.com/view/proliferation-rare-disease-rd-necessitating-novel-strategies. Accessed July 2022. 4. Sakate, R. et. al. Trends of Clinical Trials for Drug Development in Rare Diseases. Current Clinical Pharmacology. Volume 13 Issue 3. Available at https://pubmed.ncbi.nlm.nih.gov/29866013/. Accessed July 2022. 5. Adams, B. More than a quarter of rare disease trials are culled due to low patient rates: report. FierceBiotech. Available at https://www.fiercebiotech.com/cro/more-than-a-quarter-rare-disease-trials-are-culled-due-to-low-patient-rates-report. Accessed July 2022. 6. RARE Disease Facts - Global Genes. Global Genes Rare Disease Facts. Available at https://globalgenes.org/rare-disease-facts/. Accessed August 2022.