Rare diseases are a public health concern worldwide. What makes them so rare? In many cases, they affect less than 200,000 people in the United States. Globally, they impact 360 million people, surpassing the population of the world's third largest country. With viable drug treatments available for merely 5% of these indications, PRA knows that now is the future of pharmaceutical development, and fighting for a chance to live another day. We understand this fight and are proud to be part of the community, and especially their lack of treatment options. Through our new strategic partnership with Global Genes, Kakkis EveryLife Foundation: www.everylifefoundation.org, and Global Offices in 80+ countries, we are ensuring that rare disease patients are included within the clinical research ecosystem, and that their voice and perspective are heard.

Having conducted over 200 rare disease studies in the last 5 years, PRA possesses a clear understanding of the challenges and critical success factors necessary to successfully execute rare disease studies. Our relevant experience encompasses operations and project management staff have experience managing and executing rare disease studies.

The economic impact of diagnosing and managing rare diseases is significant. The journey through diagnosis and beyond due to the lack of standards of care, and cultural and health coverage decisions can become financially overwhelming. Although each rare indication affects <200,000* people globally, rare diseases collectively impact 360 million people, surpassing the population of the world's third largest country. With viable drug treatments available for merely 5% of these indications, PRA knows that now is the future of pharmaceutical development, and fighting for a chance to live another day. We understand this fight and are proud to be part of the community, and especially their lack of treatment options. Through our new strategic partnership with Global Genes, Kakkis EveryLife Foundation: www.everylifefoundation.org, and Global Offices in 80+ countries, we are ensuring that rare disease patients are included within the clinical research ecosystem, and that their voice and perspective are heard.

According to patient/caregiver respondents, in order to get a proper diagnosis, a patient typically experiences:

- 80+ visits
- 4 to 8 specialists
- 20 to 40 tests and procedures
- No. of misdiagnoses: 7 or more
- 3 to 12 months to get a proper diagnosis

Every day, millions of people throughout the world are fighting—fighting for a change in the perception of rare diseases, fighting for a voice in the development of treatments.

*In the US, a condition is considered “rare” if it affects fewer than 200,000 persons combined in a particular rare disease group.

We are committed to helping rare disease patients and emerging companies to advocate for a chance to live another day. We are ensuring that the voice of patients and their caregivers during study design and throughout the execution of clinical research processes allowing our staff to harness practical knowledge and experiences allowing our staff to engage, and empower each patient in shaping the future of rare diseases clinical development. Most importantly, we resolve to be an ally with patients, parents, families, and caregivers can't wait. A disease might be rare, but hope should not be.

The FDA approves 32.6M drugs, 334M treatments, and 320M+ solutions. The 1983 Orphan Drug Act made it possible for rare disease patients to access treatment. To date, 425 indications have been approved.

As disparate as rare disease are, patients share many common frustrations. Avoiding lengthy diagnostic episodes, finding the right treatment, a patient typically experiences: 80+ visits, 4 to 8 specialists, 20 to 40 tests and procedures, No. of misdiagnoses: 7 or more, 3 to 12 months to get a proper diagnosis, 80+ visits, 4 to 8 specialists, 20 to 40 tests and procedures, No. of misdiagnoses: 7 or more, 3 to 12 months to get a proper diagnosis.