ALEXION
AstraZeneca Rare Disease

Leading in Collaboration with the Rare Disease Ecosystem

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Rare diseases are inherently unique and not well understood, which creates a complex research environment. As a result, developing rare disease medicines requires collaboration with patients, health care professionals, advocates, investigators, pharmacists, health authorities, policymakers, and many others, who share our passion for advancing research and development to help transform lives. Our success in delivering innovation over the past 30 years would not be possible without ongoing collaboration with the rare disease ecosystem. It takes a village!

Especially in rare diseases, where patient populations are small, direct engagement with patients and caregivers is critical in helping us understand these conditions and the impact on patients' lives. This collaboration enables us to design, tailor, and execute meaningful clinical trials that meet their unique needs. Patient advocacy groups and healthcare providers, who are on the front lines with patients, are important to making these connections and providing expert perspectives to inform our research priorities and development pipelines.

Additionally, investigators, study coordinators, pharmacists and many others work tirelessly at clinical trial sites across the globe to help us enroll patients in our studies and ensure they have a positive trial experience. This close collaboration helps ensure high quality data that will advance scientific understanding and contribute to the development of new treatment options for patients.

Moke Sharma, SVP, Head of Development Operations & Quantitative Sciences, reinforces the importance of collaboration in accelerating clinical research: "Everyday counts for those living with a rare disease, and it is our responsibility to use all of the resources at our disposal to get answers quickly. Well-informed and diligently managed clinical trials are instrumental to this effort, and they would not be possible without community partners working with us toward this common purpose of improving lives."

The collective efforts of these groups, along with health authorities, payers and legislators, continues to be essential to help change the trajectory of many rare diseases. Together, we have the opportunity to further elevate the voice of rare disease patients in health policy discussions, create greater access to medicines for those in need, and advance technologies aimed at shortening the rare disease diagnostic odyssey.

The rare disease community may be small in comparison to common conditions, but it is a mighty and united front. Together, we are pushing boundaries and relentlessly following the science to serve patients and their families. Learn more about our 30 years of leadership in rare diseases by visiting our Research & Development page.