HEALTH EQUITY

ADVANCING COMPREHENSIVE CARE

A summary of Alexion's Listening & Learning Webinar on March 1, 2022

EQUITABLE ACCESS TO COMPREHENSIVE HEALTHCARE is essential for all individuals, yet for rare disease patients it remains a complex challenge. Collaboration among all stakeholders in the rare disease ecosystem is critical to advancing solutions to address the unmet needs of this patient population

MORE THAN 30 MILLION AMERICANS

NEARLY 1 IN 10 LIVE WITH A RARE DISEASE.

ON AVERAGE, IT WILL TAKE A RARE DISEASE PATIENT

4.8 YEARS + 7.3 SPECIALISTS

TO RECEIVE AN ACCURATE DIAGNOSIS[2]

NORD'S RARE DISEASE CENTERS OF EXCELLENCE is a network of 31 designated medical centers, clinics and institutions that give patients access to the best possible coordinated multi-specialty clinical care and diagnostic opportunities.

17% OF INDIVIDUALS IN 2019 HAD ALREADY RELOCATED OR WERE CONSIDERING RELOCATING TO ACCESS CARE FOR THEIR RARE DISEASE.[1]

Rare disease specialists are often concentrated to certain parts of the country, resulting in many patients needing to travel long distances to receive care.

» **Telehealth technologies** help eliminate geographic, socioeconomic, and health-related restrictions that prevent them from traveling to specialty care appointments.

"The needle needs to move. We need to improve the ways patients are able to access care."

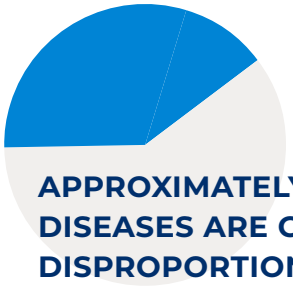
ONLY 5% OF RARE DISEASES HAVE AN FDA-APPROVED TREATMENT OPTION.[3]

» **The accelerated approval pathway** helps expedite the approval of innovative treatments for rare diseases that are too challenging to study using a traditional pathway, without compromising FDA's stringent, science-based approval standards.[4]

This includes the use of:

- **PATIENT REGISTRIES**
- **REAL-WORLD EVIDENCE**

The » **Orphan Drug Tax Credit** provides incentives for innovation and encourages companies to develop new novel rare disease therapies and explore the potential of approved therapies to treat other rare conditions.



APPROXIMATELY 80% OF RARE DISEASES ARE GENETIC AND SOME DISPROPORTIONATELY AFFECT CERTAIN RACIAL AND ETHNIC MINORITY GROUPS.[5]

Minorities are underrepresented in genome-wide association studies and clinical trials, leading to a lack of understanding about certain rare diseases. Properly understanding the genomics of diverse ethnic populations is critical to increasing the speed of diagnosis and drug development.

POLICIES TO PAY ATTENTION TO

- Newborn Screening Saves Lives Reauthorization Act
- (H. Res. 948) Resolution recognizing the challenges faced by patients of color with rare diseases
- Changes to the Orphan Drug Tax Credit under the Build Back Better PlanCures 2.0 legislation
- Speeding Therapy Access Today (STAT) Act
- Diverse and Equitable Participation in Clinical Trials Act (DEPICT) Act
- Diversifying Investigations VIA Equitable Research Studies for Everyone (DIVERSE) Trials Act

"It's about power in numbers. Together, we can use our voice and stories to amplify our core messages to Congress."

"Communities of color already fight to be included and heard in a system that is predicated on keeping them under margins. The lingering effects of historic bias manifests themselves in hurdles to care and worse health outcomes."

BEYOND HEALTH CARE > WRAPAROUND SUPPORT

- AstraZeneca's ACT (Accelerating Change Together) on Health Equity
- Rare Belonging, part of the Alexion Charitable Foundation

"Patients and their families often need things beyond medicine and what we can provide as a company."

ADDITIONAL RESOURCES

- EveryLife Foundation
- Rare Disease Diversity Coalition
- NORD
- Global Genes

FOR U.S. AUDIENCES ONLY

1. NORD. Barriers to Rare Disease Diagnosis, Care and Treatment in the US: A 30-Year Comparative Analysis. https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-Survey-Report_Infographic_FNL.pdf. Accessed February 2022. **2.** Engel PA, et al. Physician and patient perceptions regarding physician training in rare diseases: the need for stronger educational initiatives for physicians Journal of Rare Disorders 2013: Vol. 1, Issue 2. Available at http://www.journalofraredisorders.com/pub/IssuePDFs/Engel.pdf Last accessed November 2015. **3.** Global Genes. Rare Disease Facts. https://globalgenes.org/rare-disease-facts/. Accessed February 2022. **4.** The Rare Disease Company Coalition Urges Congress to Preserve and Reinforce Accelerated Approval Program to Enable Delivery of Therapies for Challenging Rare Diseases, Rare Disease Company Coalition 2021, https://www.rarecoalition.com/2021/07/29/the-rare-disease-company-coalition-urges-congress-to-preserve-and-reinforce-accelerated-approval-program-to-enable-delivery-of-therapies-for-challenging-rare-diseases/. **5.** NAC Report. NAC report: https://www.caregiving.org/rare/. Accessed February 2022.