

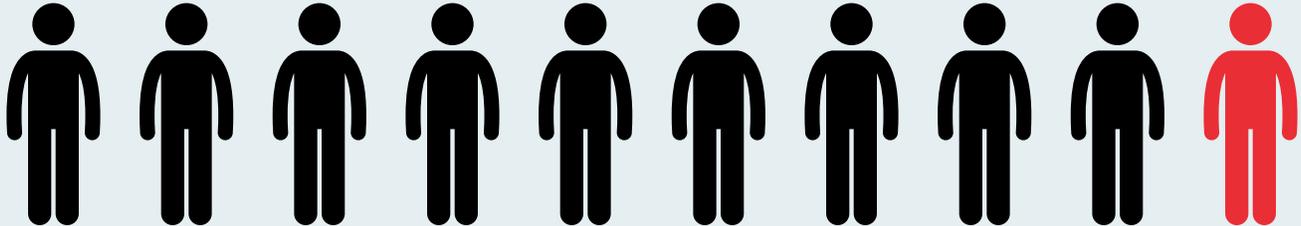


**Reducing Time to Diagnosis for  
People Living with Rare Diseases:  
A Conversation on U.S.  
Policy Opportunities**



# Executive summary

When considered as a whole, rare diseases are anything but uncommon. In fact, rare diseases impact an estimated 30 million individuals in the U.S., or one in 10 people, of all races, ethnicities and backgrounds.



The combined burden of these diseases on patients, caregivers and families, as well as on the health care system and economy, is substantial. Diagnosis of a rare disease is associated with a devastating physical and emotional toll, particularly given the disproportionate impact of rare diseases on children. Additionally, rare diseases represent a significant economic burden, with one recent study finding that **the total cost of rare diseases in the U.S. approached \$1 trillion in 2019.**

Adding to these challenges is the difficulty in identifying and diagnosing rare diseases, in part because they can mimic more common conditions. The inability to obtain a timely diagnosis continues to be a major barrier to obtaining life-altering care and improved quality of life for this community. A recent study determined that an average of 17 interactions with the U.S. health care system are needed to arrive at a proper diagnosis for a person with a rare disease. This status quo is not sustainable for patients, the rare disease community more broadly and the health care system.



Takeda is committed to reducing the burdens faced by the rare disease community, not only through the development of new treatments and potential cures, but also through efforts to advance multistakeholder solutions to reduce time to diagnosis. Based on discussions with rare disease experts and key patient group representatives, we have identified three opportunities with high potential for sustained impact:

1. Widespread and equitable access to genetic screening, including newborn screening and genomic sequencing
2. Investment in centralized and specialized rare disease care through Centers of Excellence and Networks of Care
3. Improvement in the data landscape through standardization, centralization and appropriate sharing and collaboration

In this white paper, we describe these opportunities in greater detail and why we view them as important steps to shortening the time to diagnosis for all patients with rare diseases, including those from communities historically marginalized by the health care system. We recognize that development and implementation of policy changes require the collaboration and commitment of a variety of stakeholders, and we view this paper as our contribution to advancing the conversation. Together, we can drive policy action for the benefit of people living with rare diseases and their loved ones.

# Full report

Takeda is committed to contributing in meaningful ways to improve diagnosis, care, treatment and, ultimately, the quality of life of individuals living with rare diseases. We also understand that effecting meaningful change necessitates the partnership of multiple stakeholders. Through continuous dialogue with the rare disease community, we aim to identify proposals for U.S. policy action for the benefit of people living with rare diseases and their families. To further the conversation, this paper highlights three areas of high impact for public and private collaboration. Join us in turning the vision of optimal care for patients with rare diseases into a reality. Together, we can do more.

## Expert partners interviewed in the development of this paper include:

- Amyloidosis Research Consortium (ARC)
- EveryLife Foundation for Rare Diseases (EveryLife)
- National Organization for Rare Disorders (NORD)
- Parent Project Muscular Dystrophy (PPMD)



With a significant proportion of our research and development portfolio focused on rare diseases, Takeda is working to find cures and advanced therapies for people living with rare diseases. At the same time, we understand that there is much to be done while this research is ongoing. Time to diagnosis continues to be one of the most significant hurdles for this population. A factor commonly viewed by rare disease experts as contributing to delays in diagnosis is that many symptoms of rare diseases resemble those of more common conditions, which results in missed diagnoses and misdiagnoses. Incomplete understanding of the progression of most rare diseases, short consultation times and delays in access to specialists have also been identified as challenges. Despite a growing number of diagnostic

tools and increased professional expertise, **it still takes an average of six years after symptom onset for a patient to receive an accurate diagnosis.**<sup>1</sup> Vital specialized care and expertise can be even more challenging to access for historically marginalized communities, and there is a lack of diverse representation in research studies, resulting in significant barriers to care for people of color and other underserved communities suffering from rare diseases.<sup>2</sup>

## Ensuring meaningful and sustainable improvement will require cross-stakeholder collaboration.

Patients, caregivers and their advocates must be actively involved with the medical community, academics, and private and public institutions to provide a clear understanding of the community's needs, priorities and experiences. The academic and medical communities (including clinical geneticists, primary care providers, pediatricians and other health care providers) should continue to partner with patient stakeholders and biopharmaceutical and technology companies to develop and implement new ways to reduce the time to diagnosis for patients with rare diseases. Policymakers and regulators in the U.S. also need to be engaged with all stakeholders to adapt or adopt systems and frameworks that lead to improved health outcomes for people living with rare diseases.

Although each specific rare disease affects a small percentage of the population, together rare diseases are a significant public health challenge, impacting more than one in 10 Americans.<sup>1</sup> Rare diseases do not discriminate, affecting people of all races, ethnicities, backgrounds and cultures. Beyond the individuals affected, rare diseases also create challenges for family members—particularly because about 50% of patients with rare diseases are children.<sup>1</sup>

Rare diseases also impose a substantial economic burden on the health care system more broadly. In a recent study, the EveryLife Foundation for Rare Diseases found that the total direct and indirect cost of rare diseases in the U.S. approached \$1 trillion in 2019.<sup>3</sup>



**Additionally, the study found that survey respondents experienced an average of 17 interactions with the health care system before receiving an appropriate diagnosis.<sup>3</sup>**

These interactions included visits to primary care physicians, specialists, and emergency departments, as well as hospital admissions and out-of-state trips to seek a diagnosis.<sup>3</sup> Reducing the number of health care interactions before diagnosis could not only potentially lower costs, but could also decrease the burden patients and caregivers face when navigating the complexities of the health care system.

“The country needs to understand the value of early diagnosis for this population. It does not end with the nucleus of the family; it has significant economic and financial repercussions in the health care system.”

Pam Gavin,  
Executive Vice President, NORD

We believe it is time to direct our nation's collaborative energy to shorten the time to rare disease diagnosis for the 30 million Americans and their families awaiting a healthier future. The opportunities described in this paper are those with high potential for sustained impact through meaningful public policy. While there are many challenges to reducing time to diagnosis for people living with rare diseases, the areas of focus discussed in this paper were assembled through interviews with Takeda experts in rare disease and leaders from rare disease patient organizations. As key partners in reducing time to diagnosis, this group of stakeholders is at the forefront of advocacy initiatives and policy discussions. We hope this is the beginning of an ongoing conversation with the community and welcome the input of our partners and colleagues to best effect meaningful change, advance equity and improve health outcomes.



# Widespread and equitable access to genetic screening

The first opportunity with high potential to reduce time to diagnosis is genetic screening, including newborn screening and genomic sequencing (whole genome and exome sequencing). Expanded access to genetic screening can speed diagnosis for people living with a rare disease, securing a better window of opportunity to intervene before irreversible damage to health occurs and reducing the economic burden on our health care system.

Screening newborns has shown to save lives and improve quality of life through early detection and prompt treatment. At the federal level, the Recommended Uniform Screening Panel (RUSP) sets national recommendations for the conditions that should be screened for at birth.<sup>4</sup>

**Although all states screen for a number of the recommended rare conditions, each state operates its newborn screening program independently, creating geographic variability and inequities.<sup>5</sup>**

“ Newborn screening is the closest thing we have to a silver bullet for many rare diseases. We must ensure equitable access to screening for every baby born in the U.S. ”

Annie Kennedy,  
Chief of Policy and Advocacy, EveryLife

Inclusion in the RUSP involves extensive requirements, such as the availability of appropriate tests and treatment and demonstration of benefit from early intervention.<sup>6</sup> Adding a new condition to the RUSP entails immense effort to gather appropriate evidence and present a strong case for inclusion in the national recommendations.<sup>6</sup> The EveryLife Foundation for Rare Diseases is working with several states to streamline the process for adding a new condition to the state's screening panel, following updated recommendations from the RUSP.<sup>7</sup> Such legislation is needed to ensure that every infant in every state has timely and equitable access to comprehensive screening panels that reflect national recommendations.

## Newborn screening in the U.S.

The Newborn Screening Saves Lives Act, passed by Congress in 2008, facilitated the expansion of newborn screening panels in the U.S. The Act, which must be reauthorized every five years, has improved the assessment, coordination and treatment of infants by educating and training laboratory personnel in screening programs and technologies.

Takeda supports the Newborn Screening Saves Lives Reauthorization Act of 2021 because it seeks to help states expand and improve screening programs as well as promote health care provider and patient education.<sup>8</sup>

Beyond screening for RUSP-recommended conditions at birth, other screening methods are available to the wider population, particularly for adult-onset conditions. Such screening can also provide answers early in the search for a diagnosis. Genomic sequencing, when used as a screening tool, can help confirm a diagnosis of a genetic disorder and enable health care providers to understand how an individual will respond to certain treatments.<sup>9</sup> **In a recent study, researchers at the Karolinska Institutet explored how sequencing the genome of more than 4,000 individuals during a five-year period led to a rare disease diagnosis for more than 1,200 previously undiagnosed patients.<sup>10</sup>** The study also demonstrated the value of early screening in improving clinical outcomes, as genetic counseling, prognostic information and individualized treatments became available for diagnosed individuals.<sup>10</sup> This study and other sequencing projects around the world suggest that the incorporation of both targeted genetic panels and genome sequencing in clinical practice can significantly shorten the rare disease diagnostic journey.

Our vision is a future in which genetic screening is available and accessible for all individuals and families, and health care providers are equipped and activated to drive appropriate screening. Access to early testing potentially reduces the hardship of the diagnostic odyssey and allows families to stay informed and involved in ongoing research into potential therapies. It also helps build understanding about the progression of a disease and could encourage further investments, including research and clinical trials, in previously unknown and underdiagnosed conditions. These powerful diagnostic mechanisms are transforming our ability to identify and treat rare genetic diseases.

We also believe that counseling services should be available to those undergoing testing. Counselors can help individuals and families interpret and process genetic testing results. Efforts underway in this area include the Genetic Counseling Cultural Competence Toolkit supported by the National Society of Genetic Counselors, ensuring culturally sensitive services nationwide.<sup>11</sup>

The U.S. needs robust policies to address systemic barriers and ethical considerations in screening and sequencing. These issues include privacy concerns and limited access to trained specialists and genetic counselors. Because advances in technology are rendering genetic testing increasingly affordable, public policy will need to keep pace with technology to ensure that these services are safe, reliable and accessible to all.



**50% of patients with rare diseases are children<sup>1</sup>**



# Investment in centralized and specialized rare disease care

Advancing Centers of Excellence (“Centers”) and Networks of Care (“Networks”) is a second opportunity to make a measurable impact on reducing the time to diagnosis of rare diseases. Centers house multiple specialists with complementary competencies, while Networks leverage technology to offer virtual collaboration among medical professionals. These Centers and Networks, whether specializing in rare diseases generally or in a subset of disorders, offer undiagnosed patients a direct pathway into the health care system, where targeted testing and expert medical staff help identify rare conditions more readily.<sup>12</sup> Once accurately diagnosed, patients may require the attention of multidisciplinary health care teams, often located throughout the country.

“Complex referral systems and long wait times between specialist visits can delay diagnosis. Centralized, specialized care can ease the diagnostic journey for patients and families.”

Pat Furlong,  
Founding President and CEO, PPMD

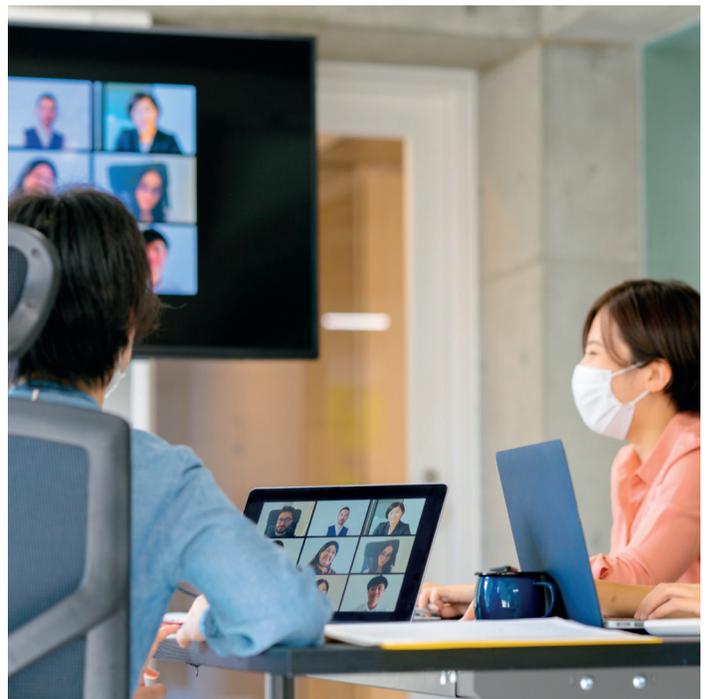
Both Centers and Networks facilitate navigation of the complex diagnosis, treatment and care landscape and improve communication across specialties. They also enhance efficiencies within the health care system and relieve patients and their families of the burden of coordinating care across multiple medical specialists and venues. This is especially important to facilitate continuity of care and access to health care services for patients in underserved and rural communities. **Centralization also leads to improved health outcomes and quality of life through standardized care practices and guidelines and volume-based experience: the more patients seen, the more expertise available to improve care.**<sup>13</sup> Centers and Networks combine medical expertise and knowledge at a national level to provide holistic care to people living with a rare disease.

## Rare Disease Institute becomes U.S. Center of Excellence for rare genetic diseases

In 2017, Children’s National Health System announced it had formed the Children’s National Rare Disease Institute, a first-of-its-kind Center of Excellence for rare genetic diseases in both children and adults. The center now has about 8,500 visitors annually and is home to one of the largest clinical genetics programs in the U.S.<sup>14</sup>

This effort to improve patient care builds on the ongoing efforts of the cystic fibrosis<sup>15</sup> and Duchenne muscular dystrophy<sup>16</sup> communities to establish and recognize Centers of Excellence to provide holistic, multidisciplinary, networked care.

One of the components of the Rare Disease Institute Center of Excellence project is a Rare Disease Clinical Activity Protocol Program (Rare-CAP) aimed at standardizing processes regarding diagnosis and care. Takeda is proud to support this effort.



Furthermore, centralized expertise facilitates the study of specific disorders. The National Institutes of Health Centers of Excellence Rare Diseases Clinical Research Network (NIH Network), established in 2003, promotes rare disease clinical trials and other studies.<sup>17</sup> **Collaborations through this network have focused on some 240 rare diseases involving more than 40,000 research participants, leading to greater understanding of these conditions.**<sup>17</sup>

Improvements in monitoring of amyotrophic lateral sclerosis (ALS or Lou Gehrig's disease) to track disease progression and determine treatment effectiveness is an example of the NIH Network's contributions. Their work also includes advances in gene editing mechanisms to prevent or reverse Hunter syndrome, a rare metabolic disorder.<sup>17</sup> The NIH Network continues to focus on addressing unmet needs in order to advance clinical trials and ultimately treatment.

Centers are not a new concept in the U.S.; federal agencies and patient groups have worked to establish, designate and expand them for the past two decades. Recruitment and training of medical specialists, including geneticists and genetic counselors, is also necessary. Some patient groups, such as the Amyloidosis Research Consortium, already offer extensive training and materials for health care professionals to increase their competencies in rare diseases.<sup>18</sup> Further efforts to standardize education in rare diseases, such as biomarker interpretation across medical specialties, will improve diagnostic practices and care for this population. Continuing to advance clinical research on rare diseases, and incorporating diverse representation in clinical trials, is also vital to ensure we understand these conditions, their impact on diverse communities and potential treatments and cures.



**It still takes an average of six years after symptom onset for a patient to receive an accurate diagnosis<sup>1</sup>**

Our vision is a future in which people with rare diseases can access specialized, coordinated care no matter where they live in the U.S. Accomplishing this will require investment in national guidelines that set the minimum threshold to certify Centers, as well as governance for insurance providers that requires coverage of care at these Centers at in-network cost-sharing rates across state lines.

Centers and Networks have already proven to be beneficial in improving patients' health outcomes, advancing research and reducing the burden on health systems. These activities need to be scaled to support improved diagnosis by building an appropriate workforce and ensuring equitable insurance coverage so that all individuals living with a rare disease in the U.S. can benefit from these crucial advances.



# Improvement in the data landscape

Third, data standardization, centralization and sharing are essential to reduce time to diagnosis. **Given the small number of individuals affected by any one rare disease, as well as the lack of centralization and standardization of the limited data that do exist, there is a dearth of high-quality data to support disease diagnosis.** Data centralization and sharing expand the value of existing data on rare diseases, helping to overcome these challenges.

“ It’s hard to overemphasize the importance of rare disease data. We must prioritize data generation, sharing and standardization if we are going to solve the major challenges that the rare disease community faces today. ”

Isabelle Lousada,  
Founder and CEO, ARC

Public-private partnerships are paving the way in analyzing consolidated health information to develop artificial intelligence-based algorithms that support identification and differential diagnoses of rare diseases. Symptoms of a rare disease often are not specific to one condition alone, appear over time, and are treated by different specialists until someone recognizes a pattern. These algorithms can more readily and systematically help identify patterns that are indicative of the onset of a rare disease. They can also support physicians in interpreting laboratory and imaging data. Furthermore, pooled data from natural history studies and real-world data collection facilitate understanding of disease progression and how a disease manifests across different populations. Better understanding of the disease, including its symptoms, causes and risk factors, not only leads to reduced time to diagnosis but also advances treatment. For example, robust comprehension of the natural progression of a disease is critical to clinical development and approval of new treatments by providing comparators to demonstrate efficacy of an experimental therapy.

Efforts to encourage rare disease data sharing in the U.S. are included in several policy frameworks and agency programs. In 2019, the U.S. Food and Drug Administration launched the Rare Disease Cures Accelerator-Data and Analytics Platform to provide standards for data collection and encourage sharing of patient-level data.<sup>19</sup>

Patient groups play an integral role in supporting and promoting compliant and rapid data sharing through common platforms. Successful examples include the work of the Friedreich’s Ataxia Research Alliance<sup>20</sup> and Parent Project Muscular Dystrophy<sup>21</sup> to curate high-quality patient registries for use by the global rare disease community. These groups support essential progress by enabling researchers and clinicians to appropriately access data to better understand these diseases. They also serve as models for other patient groups to follow when investing in the development of treatments and cures.

Our vision is a future in which patient advocacy groups work in collaboration with other health care stakeholders to drive and manage high-quality, robust patient registries with centralized and standardized health data. Focused policy solutions can play an important role in incentivizing collaboration over competition by facilitating efforts toward data standardization and communication across data sets, enforcing and upgrading existing requirements for data security, and improving and streamlining the consent process during data collection. Given the limited availability of rare disease data, a collaborative approach to this challenge is imperative to improve diagnosis, care pathways and quality of life for individuals living with rare diseases.

## **Accurate and specific diagnosis codes facilitate research on rare diseases**

Another important barrier to the collection and interpretation of rare disease data is the fact that many rare diseases have not been assigned an ICD-10-CM (diagnosis) code and conditions are often misclassified, thereby increasing the barriers for data tracking and collection. Advocating for the Centers for Disease Control and Prevention to expand codes to appropriately classify and track rare diseases in a timely manner can help address these limitations and increase availability of reliable, disease-specific data in the U.S.

# Call to action

Collaboration among health care stakeholders to effect fast and meaningful change is possible. From streamlining review and approval processes, to speeding clinical research, to system-wide initiatives that increase access to telemedicine, the COVID-19 pandemic has shown how the health care sector can adapt to provide solutions to improve care. Throughout the pandemic, Americans have witnessed the public and private sectors demonstrate agility and responsiveness previously unimaginable. We need to continue to leverage the flexibility shown by the health care system for the benefit of other communities, including that of individuals living with rare diseases.

This paper explored three high-impact opportunities for policy intervention:

- Widespread and equitable access to genetic screening
- Investment in centralized and specialized rare disease care
- Improvement in the data landscape to reduce time to diagnosis



**These opportunities, independently and jointly, help reduce missed diagnoses and misdiagnoses by shortening the diagnostic odyssey.** Comprehensive and targeted screening allows for earlier diagnosis and potential treatment of a rare disease. Centralizing care in a Center or Network provides a point of entry into the health care system for the undiagnosed or misdiagnosed patient with a rare disease. Centers and Networks also facilitate standardized practices to improve health outcomes and increase access to coordinated care. Lastly, increasing the utility of health data can reduce time to diagnosis and improve health outcomes through better mapping of natural disease progression, identifying symptom patterns of rare diseases, and unearthing the true burden of rare diseases in the community. Importantly, **these efforts to improve access to accurate diagnoses and specialized care are even more consequential for marginalized and underserved communities experiencing additional significant barriers to care.**

Ongoing dialogue with the rare disease community and across multiple stakeholders, including policymakers, regulators, payers, providers, academics, advocacy groups, patients and caregivers, will advance existing policies and lead to new proposals for U.S. policy action for the benefit of all people living with rare disease and their families.

Takeda is committed to working with the rare disease community and policymakers to reduce the time to diagnosis, advance equity and improve health outcomes. **With more than 7,000 identified rare diseases, affecting an estimated 30 million Americans, the time to improve diagnosis, care, and quality of life for this patient community is now.** Together, we can do more.

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