PARTNERSHIP TO FIGHT CHRONIC DISEASE

U.S. PATIENTS WITH RARE DISEASES BENEFIT FROM EARLIER ACCESS TO TREATMENT COMPARED TO YEARS OF DELAY FACED BY U.K. PATIENTS

POLICY MAKERS MUST REJECT PROPOSALS TO IMPORT U.K. STANDARDS THAT DEVALUE OLDER ADULTS AND PEOPLE LIVING WITH CHRONIC CONDITIONS THROUGH "MOST FAVORED NATION" POLICIES.

SCIENTIFIC BREAKTHROUGHS ARE LEADING TO UNPRECEDENTED ADVANCES IN TREATMENT OF RARE DISEASES, AND THE U.S. CONTINUES TO LEAD THE WAY.[1]

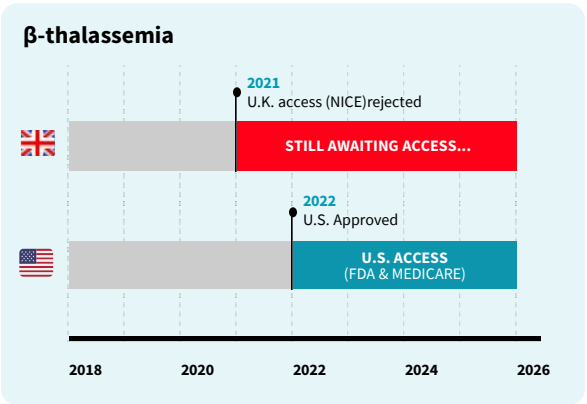
IN NATIONS LIKE THE U.K., PATIENT ACCESS OFTEN LAGS FAR BEHIND THE U.S. AND EVEN AFTER REGULATORY APPROVAL, NATIONAL HEALTH AGENCIES CREATE ADDED HURDLES.[2]

The National Institute for Health and Care Excellence (NICE) - the U.K. agency that makes reimbursement recommendations to the government – imposes cost-effectiveness standards like the Quality-Adjusted Life Year (QALY) before granting approval, causing access delays and restrictions.[3] Adoption of U.K. metrics through "Most Favored Nation" pricing proposals would effectively apply the same low value to the lives of American patients and families navigating debilitating rare and genetic diseases that are often irreversibly progressive and ultimately fatal.

U.K. PATIENTS WITH RARE DISEASE STILL DENIED ACCESS TO BREAKTHROUGH AVAILABLE IN THE U.S. SINCE 2022

U.K. Rejects Breakthrough Gene Therapy for Patients with Rare Blood Disorder

U.S. patients have had broad access to Zynteglo (betibeglogene autotemcel) to treat β-thallassemia (a rare, inherited blood disorder that, without treatment, requires lifelong transfusions) since 2022, when the therapy was approved by FDA and covered by insurers.[4] In contrast, U.K. patients have faced years of delays and restrictions due to the QALY-based thresholds employed by NICE.[5] Following a negative appraisal by NICE in 2021, the manufacturer of Zynteglo announced that it would withdraw Zynteglo from Europe.[6,7]



FIVE WAYS THE U.K. SYSTEMATICALLY BLOCKS PATIENT ACCESS TO NEW RARE DISEASE TREATMENTS

1. **Uses QALYs to undervalue patients by assigning a lower value to years of life for patients with rare diseases.** Consequently, rare disease medicines that extend or improve patients' lives often fail to meet NICE's threshold for approval, even despite NICE's attempts to mitigate this challenge through specialized pathways that allow higher QALY thresholds for certain rare disease treatments.

   Case in point: Because NICE's assessment of Vosoritide, a treatment for children with achondroplasia, the most common form of dwarfism, used low QALY-based cost effectiveness thresholds, the manufacturer was forced to withdraw the product from review in the U.K., leaving children in England without routine access to the treatment.[8]

2. **Demands unnecessary additional evidence after clinical benefit has already been demonstrated, often requiring larger or longer-term studies that are infeasible in very small patient populations.** This delays patient access by forcing them to wait years until longer-term results are available – time that many patients do not have.

   Case in point: Spinraza for spinal muscular atrophy was initially only made available to a subset of patients until years of additional real-world evidence could be collected, delaying access for at least 3 years.[9]

3. **Ignores outcomes that are important to patients living with rare diseases like fatigue, neuropathy, and slower disease progression, as well as quality-of-life benefits like ability to return to work or care for family.** As a result, NICE systematically undervalues medicines for these patients.

   Case in point: Early NICE committee discussions on Orkambi for cystic fibrosis failed to fully capture important benefits to patients in areas such as independence, education, employment and family burden, contributing to a 4-year delay in patient access despite strong evidence of transformational clinical benefit.[10]

4. **Fails to value long-term benefits of rare disease medicines, resulting in prolonged reassessments and delayed patient access even when evidence suggests therapies can be life changing for patients.**

   Case in point: Translarna for Duchenne muscular dystrophy initially secured regulatory approval in 2014 in the U.K., but the therapy underwent nearly 10 years of reassessment before securing routine coverage in 2023 due to NICE's failure to recognize its benefits like slowed disease progression and preservation of motor function for these patients.[11]

5. **Imposes narrow coverage restrictions, even when new evidence shows broader use could be beneficial.** In the U.S., by contrast, rare disease medicines are commonly covered for many medically beneficial uses beyond those in FDA-approved labeling.

   Case in point: NICE's Givinostat, also for Duchenne muscular dystrophy, underwent an appraisal process that lasted almost 2 years. When NICE finally granted coverage, it limited access only to patients who are still able to walk, leaving patients who couldn't without access despite the progressive and irreversible nature of the disease.[12]

Learn more about the unintended consequences of "Most Favored Nation" at www.fightchronicdisease.org/MFN

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