

UNITED KINGDOM

Between 2002 and 2014,

40%

of medicines to treat orphan conditions were **rejected for coverage** in the U.K.

U.K. patients are

2/3 less likely

to receive PD-1 inhibitors – an innovative new cancer treatment–compared to U.S. patients ²

Between 2007 and 2017, nearly

80%

of cancer treatments reviewed by U.K. health officials had some form of access restriction. ²

Access Restrictions in the U.K.

Rigid assessments of treatment value, government-driven decisions on formularies and coverage, and insufficient health care budgets have long stood in the way of U.K. patients who need access to the latest innovative medicines.

The high rate of rejections by the National Institute for Health and Care Excellence (NICE), the group that advises the National Health Service (NHS) on coverage decisions and makes decisions based on a cost-effectiveness threshold of between £20,000 and £30,000 (\$25,700 - \$38,560) per quality-adjusted life year (QALY), is one major reason why U.K. patients are often denied access to new medicines.

That's because medicines exceeding NICE's cost-per-QALY threshold are not deemed cost effective. This leaves the patients who need treatments that carry high development costs or treat small populations high and dry. In fact, for every 100 patients in peer countries who get access to a new medicine in its first year of launch, just 18 patients in the U.K. are given access to these treatments.

"There is a single entity in the UK – that doesn't seem to have accountability to anyone – that gets to decide the monetary value for a person's health."

Parent of a child with cystic fibrosis in the U.K.⁶



Although NICE conceded that Spinraza "provided a substantial clinical benefit," the committee ultimately recommended against covering Spinraza due to its failure to meet cost effectiveness standards. Mencia de Lemus Belmonte, President of SMA Europe, was dismayed at the decision, "It's so hard for me to believe that a national agency of a European country that has a sound economy would reject such an important drug for such a devastating disease for economic reasons."

S Mardiguian, M Stefanidou, et al. Trends and key decision drivers for rejecting an orphan drug submission across five different HTA agencies. (2014).

² IQVIA. Global Oncology Trends 2018, May 2018.

³ Drummond, M. and Sorenson, C. Nasty or Nice? A Perspective on the Use of Health Technology Assessment in the United Kingdom. Value in Health 2009; 12(S2).

⁴ OHE analysis of 61 medicines launched in the UK since 2007 compared to 16 countries (2014).

⁵ Esiason, Gunnar. Why does the UK put a lower value on the life of a CF patient than the rest of the world? October 17, 2018.

⁶ Luxner, Larry. 'SMA groups outraged over UK rejection of Spiranza coverage as too expensive.' SMA News Today. August 2018.