REGIONAL VARIATIONS IN APPRAISAL AND UPTAKE OF NEW TREATMENTS FOR ULTRA RARE DISEASES IN THE UK: A CASE STUDY OF ATALUREN FOR nmDMD

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Objectives

The UK is often thought of as one single-payer market. The National Institute of Health and Care Excellence (NICE) is an internationally renowned organisation leading innovation in the assessment of new treatments for ultra-rare diseases, with the relatively recent development of its Highly Specialised Technology (HST) programme and the facility to provide conditional reimbursement via a managed access agreements (MAA). However, NICE HST guidance only applies to England, which represents 80% of the UK population.

We describe regional variations in gaining reimbursement for ataluren, which is licensed for the treatment of nonsense-mutation Duchenne muscular dystrophy (nmDMD)[1]. There are currently estimated to be 85 nmDMD patients eligible for ataluren in the UK, of which 72 are in England.

Methods

We reviewed the health technology assessment (HTA) processes used to evaluate the clinical efficacy and cost-effectiveness of ataluren across the UK, and the associated timelines to reimbursement and patient access. The process and reimbursement timelines for England, Scotland, Wales, Northern Ireland (NI) and the Isle of Man (IoM) were reviewed.

Results

Conditional marketing authorisation was granted by the European Medicines Agency (EMA) in July 2014[1]. Over a year later the first patient received NHS-funded ataluren in Scotland via an Individual Patient Treatment Request [IPTR] (Figure 1). Individual funding requests elsewhere in the UK were unsuccessful.

Following a 13-month NICE review, positive NICE guidance was published under the conditions of a multi-stakeholder 5-year MAA agreed between PTC, NICE, NHS England, the NorthStar clinician network, and patient associations Muscular Dystrophy UK and Action Duchenne[2].

NI and Wales endorsed NICE guidance under a patient access scheme (PAS). The first patient treated was in Belfast in July 2016. There was only one patient in IoM, which normally follows NICE guidance. Initially IoM did not grant reimbursement following the NICE decision, but finally did agree to fund in November 2016. Despite negative guidance from the Scottish Medicines Consortium (SMC) after a shorter 5-month appraisal process, ataluren was reimbursed by NHS Scotland with a PAS.

Over this entire period patient groups and families implemented highly active national and regional political lobbying campaigns via social media, parliamentary questions and rallies.

Figure 1: Timeline for gaining reimbursement of ataluren in the UK

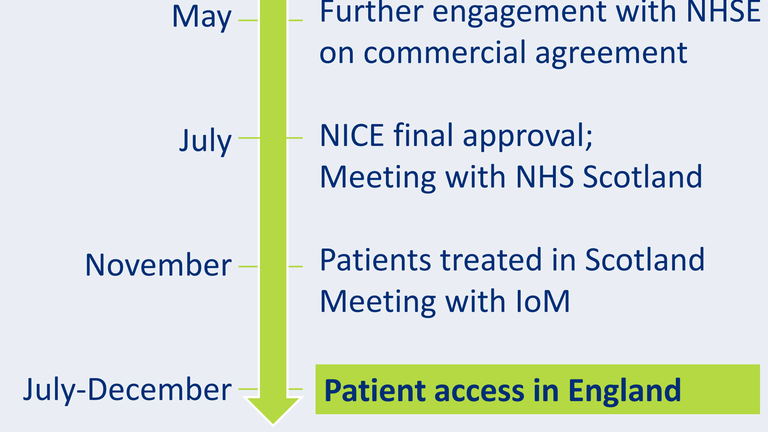
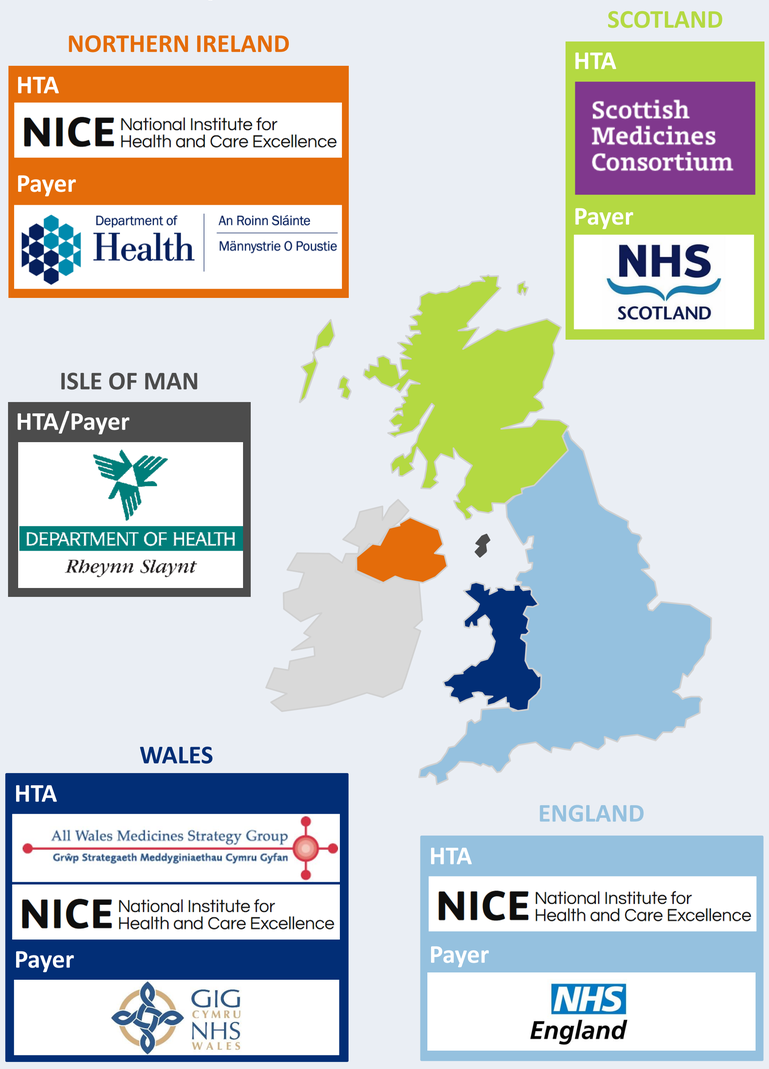


Figure 2: Healthcare organisations involved in the reimbursement process



Discussion and Conclusions

Rare genetic conditions can result in a disproportionate cost to small markets. For example, for nmDMD, there are 5 patients in NI, 1 in IoM, 1 in Wales and 6 in Scotland. This can increase the challenge in gaining reimbursement for therapies for ultra-rare diseases.

Market access in the UK does not start and stop at NICE or SMC. Through the different formal submission processes, patient and political lobbying were instrumental in achieving reimbursement outcomes across the UK. MAAs in England are enabling access to some innovative treatments but other regions do not have fit-for-purpose HTA processes for assessing products for ultra-rare conditions, as recognised by the recent Montgomery review in Scotland[3]. The decision-making power of NICE and SMC in reviewing high-cost first-in-class ultra-orphan treatments is no longer clear in a regulatory and reimbursement environment where EMA assesses safety and efficacy whilst the NHS assesses willingness to pay. The UK consists of 5 distinct regions, each with one or two decision makers, including HTA bodies and payers (Figure 2). Whilst all nmDMD patients now have funded treatment across the UK, reimbursement processes need to be improved to avoid unintended discrimination against patients with other ultra-rare diseases.

Critical success factors for the reimbursement of ultra-rare diseases

- Engage with HTA bodies early to ensure topics are prioritised where there is a fit-for-purpose process
- Be persistent in seeking engagement with payers in advance of HTA guidance
- Have a strong patient and political advocacy engagement plan
- Consider a managed access agreement in advance of any submission to NICE

References:

1. Ataluren (brand name Translarna) is indicated for the treatment of nonsense mutation DMD in patients aged 5 years and older who are ambulatory. European Medicines Agency. European Public Assessment Report for ataluren (Translarna) EMA/369266/2014. 2014.
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