Is there equity of access to highly specialised treatments between England and Northern Ireland?

HTA340

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Background

The UK has three HTA bodies for its constituent countries: NICE (England), SMC (Scotland) and AWMSG (Wales). However, the UK's smallest constituent country Northern Ireland (NI), does not have its own HTA body, but instead it typically adopts decisions made by NICE as policy.

In February 2019, the Health and Social Care (HSC) Board announced changes to the Managed Entry of New Medicines in NI, including mechanisms for commissioning therapies recommended via the National Institute for Health and Care Excellence (NICE) Highly Specialised Technologies (HST) program.[1] Herein, we assess the time taken for therapies recommended via the HST program to be added to the NI formulary.[2,3]

Methods

Managed Entry Decisions made by the HSC Board for therapies recommended via the NICE HST program were identified on the NI Formulary and the relevant data extracted in June 2023.

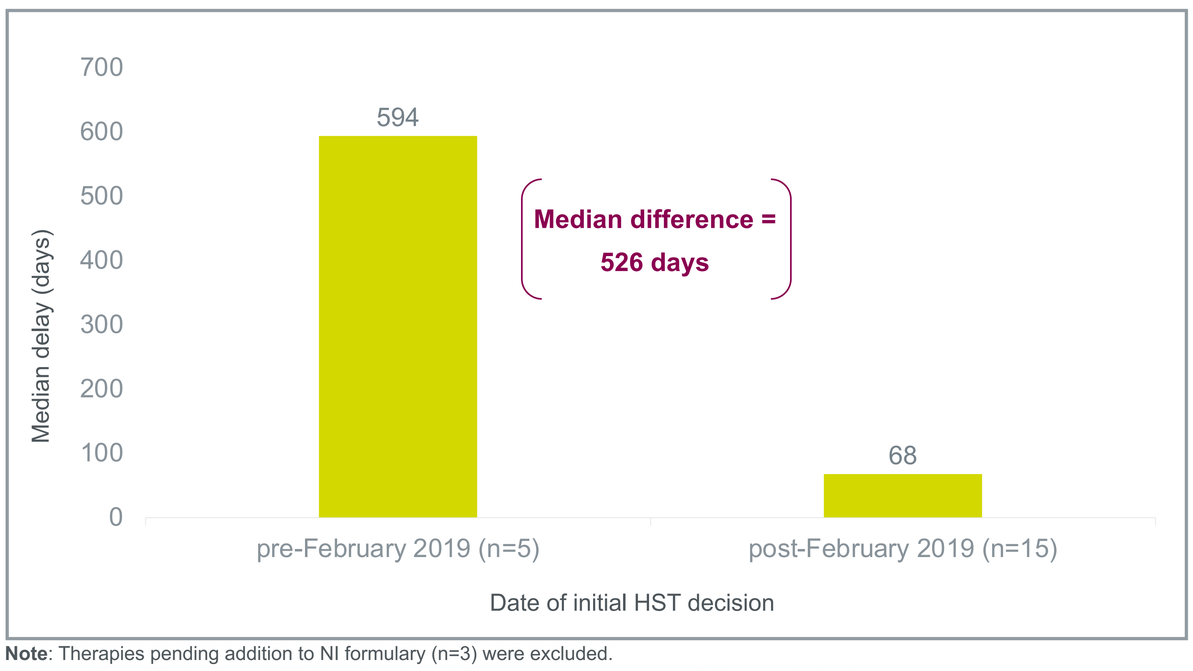
Results

Of the 23 therapy/indication pairings recommended via the HST, 20 corresponding additions to the NI formulary were identified. Three additional decisions were pending for NICE HST recommendations, i.e., onasemnogene abeparvovec, eladocagene exuparvovec, and lumasiran which had been made since April 2023 (Table 1). One therapy/indication pairing (cerliponase alfa/neuronal CLN2) was recommended with managed access by NICE. As part of the entry to the NI formulary based on the HST decision, cerliponase alfa was accepted for use in NI only if the conditions in the managed access agreement were followed.

The median number of HST-related additions to the NI formulary annually from 2016–2023 was 2 (range: 0-7). The median time for addition of therapy/indication pairings to the NI formulary was longer for therapies recommended via the HST pre- vs. post-February 2019 (594 days vs. 68 days, respectively) (Figure 1)

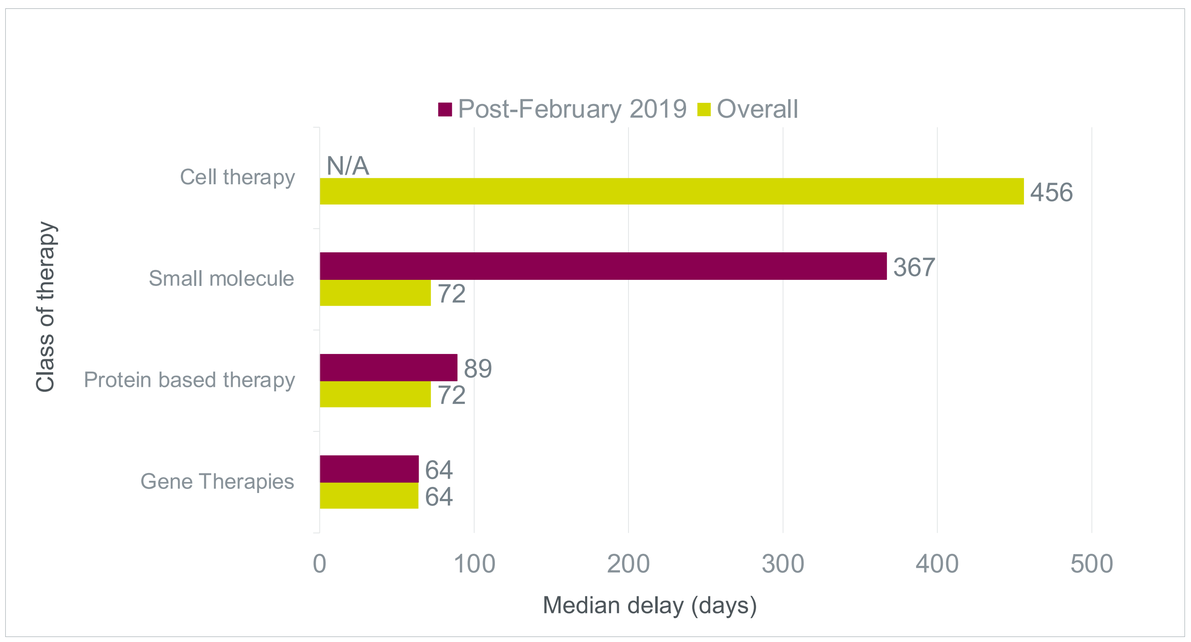
Overall, the median duration of delay was longest for cell therapies (n=1; 456 days), followed by small molecules (n=6; 72 days), protein-based therapies (n=7; 72 days) and gene therapies (n=6; 64 days) However, when analyses were restricted to therapy:indication pairings recommended post-February 2019, median duration of delay was independent of treatment type (Figure 2).

Figure 1. Median delay between HST decision and addition to NI formulary pre- vs. post HSC Board announcement for the Managed Entry of New Medicines in NI



Note: Therapies pending addition to NI formulary (n=3) were excluded.
HSC: Health and Social Care; HST: High Specialised Technologies; n: number; NI: Northern Ireland

Figure 2. Median duration of delay from initial HST decision to addition to NI formulary by class of therapy



Note: For cell therapy, no data were available post-Feb. 2019. Therapies pending addition to NI formulary (n=3) were excluded.
Feb: February; HST: High Specialised Technologies; N/A: Not available; n: number; NI: Northern Ireland

Table 1. Summary of decisions made by NICE HST and subsequent addition to NI formulary (n=23)

Therapy/indication Pairing	Class of Therapy	NICE Decision Date	NI formulary date	Difference (days)
Eculizumab (Soliris®)/Atypical haemolytic uraemic syndrome	Protein-based therapy	28/01/2015	13/09/2016	594
Migalastat (Galafold®)/Fabry disease	Small molecule	22/02/2017	09/05/2019	806
Eliglustat (Cerdelga®)/Type 1 Gaucher disease	Small molecule	28/06/2017	09/05/2019	680
Strimvelis/Adenosine deaminase deficiency	Cell therapy	07/02/2018	09/05/2019	456
Burosumab (Crysvita®)/XLH	Protein-based therapy	10/10/2018	09/05/2019	211
Inotersen (Tegsedi®)/Stage 1 and 2 polyneuropathy	Gene therapy	22/05/2019	23/08/2019	93
Patisiran (Onpattro®)/Hereditary transthyretin amyloidosis	Gene therapy	14/08/2019	10/10/2019	57
Voretigene neparvovec (Luxturna®)/RPE65-mediated inherited retinal dystrophies	Protein-based therapy	09/10/2019	17/12/2019	69
Cerliponase alfa (Brineura®)/Neuronal CLN2*	Protein-based therapy	27/11/2019	24/02/2020	89
Volanesorsen (Waylivra®)/Familial chylomicronaemia syndrome	Gene therapy	21/10/2020	08/12/2022	48
Metreleptin (Myalepta®)/Leptin deficiency complications	Protein-based therapy	24/02/2021	12/04/2021	47
Onasemnogene abeparvovec (Zolgensma®)/5q SMA	Gene therapy	07/07/2021	15/09/2021	70
Givosiran (Givlaari®)/AHP	Gene therapy	24/11/2021	0/03/2022	104
Odevixibat (Bylvay®)/PFIC	Small molecule	22/02/2022	07/04/2022	44
Atidarsagene autotemcel (Libmeldy®/ metachromatic leukodystrophy with mutations in the ARSA gene	Gene therapy	28/03/2022	12/05/2022	45
Elosulfase alfa (Vimizim®)/MPS4A	Protein-based therapy	20/04/2022	06/06/2022	47
Selumetinib (Koselugo®)/Symptomatic and inoperable PN	Small molecule	05/05/2022	20/07/2022	76
Setmelanotide (Imcivree®)/Obesity and controlling hunger	Small molecule	06/07/2022	12/09/2022	68
Ataluren (Translarna®)/Duchenne muscular dystrophy	Small molecule	22/02/2023	17/04/2023	54
Asfotase alfa (Strensiq®)/Paediatric-onset hypophosphatasia	Protein-based therapy	01/03/2023	12/05/2023	72
Eladocagene exuparvovec/TBC	Gene therapy	19/04/2023	TBC	-
Lumasiran/TBC	Gene therapy	19/04/2023	TBC	-
Onasemnogene abeparvovec (Zolgensma®)/TBC	Gene therapy	19/04/2023	TBC	-

Feb. 2019

*Recommended subject to terms of a managed access agreement by NICE

5q: Chromosome 5q; AHP: Acute hepatic porphyria; ARSA: arylsulphatase A; CLN2: ceroid lipofuscinosis type 2; Feb: February; MPS4A; mucopolysaccharidosis type 4A; PFIC: Progressive familial intrahepatic cholestasis; PN: Plexiform neurofibromas; RPE65: Retinal pigment epithelium-specific 65 kDa protein; SMA: Spinal muscular atrophy; TBC: To be confirmed; XLH: X-linked hypophosphataemia

Conclusions

- The findings from this study indicate that the timelines for the endorsement of high-cost, innovative medicines by the HSC Board in NI have been reduced. The delay from NICE HST decision to addition to the NI formulary has been streamlined to around 2 months since the changes implemented in 2019.
- However, it is also important to note that all HST-related entries to the NI formulary are caveated with the following wording: "Where infrastructure is in place and the Service has capacity, interim commissioning of this drug is accepted on a cost-per-case basis". Therefore, patient access to HST therapies may be impacted by logistical limitations, variations in Health Service provision, or budgetary constraints. As such, pharmaceutical companies should consider conducting additional regional activities to ensure that products recommended via the HST are made available for patients in NI.

References: 1. HSC Board – Letter to Trusts February 11, 2019; Managed entry of new medicines February 2019. 2. NICE HST Published Guidance. Available at: https://www.nice.org.uk/guidance/published?ngt=Highly%20specialised%20technologies%20guidance&ndt=Guidance. Accessed: June 2023. 3. Northern Ireland Formulary, Managed Entry Decisions. Available at: https://niformulary.hscni.net/managed-entry/managed-entry-decisions/. Accessed: June 2023.

Abbreviations: 5q: Chromosome 5q; AHP: Acute hepatic porphyria; ARSA: arylsulphatase A; CLN2: ceroid lipofuscinosis type 2; HSC: Health and Social Care; HST: Highly Specialised Technologies; MPS4A; mucopolysaccharidosis type 4A; N/A: Not Available; NI: Northern Ireland; NICE: National Institute for Health and Care Excellence PFIC: Progressive familial intrahepatic cholestasis; PN: Plexiform neurofibromas; RPE65: Retinal pigment epithelium-specific 65 kDa protein ;SMA: Spinal muscular atrophy; TBC: To be confirmed; XLH: X-linked hypophosphataemia

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