Keeping the ROI window open

A new major market access challenge?

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Background

- Pharmaceutical product lifecycles are normally characterised by protracted clinical development followed by marketing under exclusivity/patent protection during which returns on investment (ROI) can be realised
- However, we are entering an era of transformational therapies with curative potential (e.g. gene and CAR-T cell therapies) for whom the window to realise ROI may be greatly curtailed
- This also impacts emerging new standard-of-care which could rapidly become superseded. Nusinersen is the first and only EC-approved therapy (2017) to treat spinal muscular atrophy (SMA), a rare and fatal genetic disease
- Nusinersen has demonstrated transformation benefits for patients but requires chronic treatment. However, a potentially-curative gene therapy for SMA, onasemnogene-abeparvovec-xioi, threatens to surpass nusinersen, first launched in 2019 (US)
- This research aims to determine whether the reimbursement strategy and payer/HTA responses for nusinersen have been impacted by the impending launch of a potentially-curative competitor

Methods

- Publicly-available HTA appraisal documents for nusinersen from 20 countries including the EU5, Nordics, Australia and Canada were identified, and key information extracted

Results

- HTA reports from 20 countries for nusinersen were extracted, which were all were positive (except in Ireland), despite the complexity of the disease state, lack of long-term supportive data, and high cost of therapy
- Recommendations were limited to SMA subtypes (11%: type 1; 16%: types 1-2; 42%: types 1-3; and 32%: other restrictions/conditions)
- In the EU markets, mean delay from marketing authorisation to positive HTA recommendation was only 389 days (120-776 days)

Table 1: HTA/P&R outcomes of Spinraza (EC-approved: 30/05/2017)

Country	HTA body / P&R decision maker	Appraisal/decision date	Delay from European MA to reimbursement (days)	HTA outcome
Argentina	MoHSD	09/08/2019	–	Restricted (types 1,2,3a)
Australia	PBAC	20/04/2018	–	Restricted (types 1-3a, <18 years)
Brazil	CONITEC	25/04/2019	–	Restricted (only type 1)
Canada	CADTH	01/03/2019	–	Conditional (price drop)
Croatia	HZZO	15/07/2019	776	Approved
Denmark	DMC	30/05/2018	365	Restricted (types 1&2)
England	NICE	15/08/2018	764	Recommended (types 1-3)
Finland	PALKO	15/03/2018	289	Restricted (<18 years)
France	HAS	31/01/2018	246	ASMR III (types 1&2)
Germany	G-BA	21/12/2017	205	Added benefit (types 1&2)
Ireland	NCPE	19/12/2017	203	Rejected
Italy	AIFA	27/09/2017	120	Restricted (types 1-3)
Norway	NoMA	12/02/2018	258	Restricted (<18 years)
Poland	AOTMiT	01/01/2010	581	Approved (types 1-3)
Portugal	Infarmed	20/12/2018	569	Approved (types 1-3)
Romania	MoH	01/04/2019	671	Approved
Scotland	SMC	07/05/2018	311	Restricted (only type 1)
South Korea	MFDS	01/08/2018	–	Approved (unclear)
Spain	DGFPS	02/03/2018	276	Restricted (unclear)
Sweden	NT	20/12/2017	204	Restricted (types 1,2,3a)

Conclusions

- Nusinersen has successfully translated marketing authorisation into rapid reimbursement across a wide range of payer bodies
- However, restrictions and discounts have been necessary to facilitate this, such flexibility may have been driven by the impending launch of a competing potentially-curative gene therapy
- As more potentially-curative therapies come to market, such approaches may become increasingly commonplace among recent entrants to maximise ROI under time-limiting circumstances

AIFA: Agenzia Italiana del Farmaco; AOTMiT: Agencja Oceny Technologii Medycznych I Taryfikacji; CADTH: Canadian Agency for Drugs and Technologies in Health; CAR-T: Chimeric antigen receptor T cells; CONITEC: Comissão Nacional De Incorporação De Tecnologias No Sistema Único De Saúde; DGFPS: Dirección General de Farmacia y Productos Sanitarios; DMC: Danish Medicines Council; EC: European Commission; G-BA: Gemeinsamer Bundesausschuss; HAS: Haute Autorité de Santé; HTA: Health technology assessment; HZZO: Croatian Institute for Health Insurance; MA: Marketing authorisation; MFDS: Ministry of Food and Drug Safety; MoH: Ministry of Health; MoHSD: Ministry of Health and Social Development; NCPE: National Centre for Pharmacoeconomics; NICE: National Institute for Health and Care Excellence; NoMA: Norwegian Medicines Agency; NT: County councils' group on new drug therapies; PALKO: Ministry of Social Affairs and Health; PBAC: Pharmaceutical Benefits Advisory Committee; P&R: Pricing and reimbursement; ROI: Return on investment; SMA: Spinal muscular atrophy; US: United States

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