

Should Gene Therapies be exempt from HTA?

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What do patients want?

1 in 17 people have a rare disorder

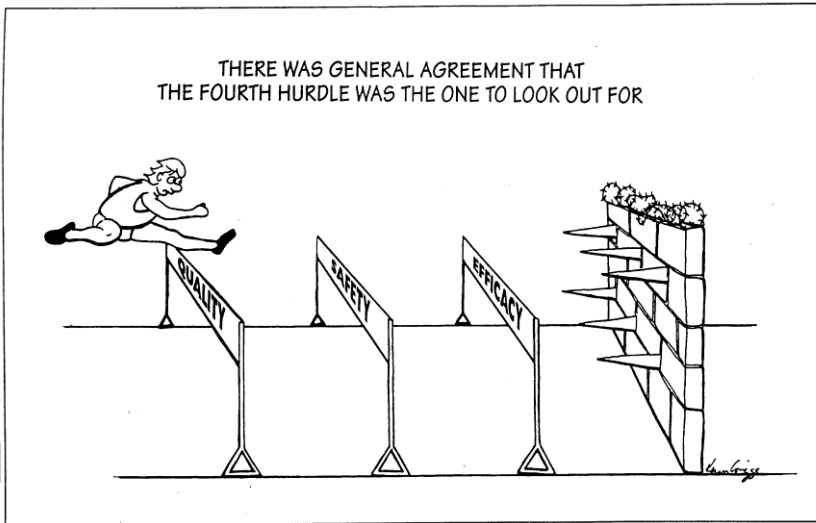
80% of rare disorders have a genetic component

95% of rare disorders do not have a licensed treatment

... and I realised that there is something worse than there being no treatment, that is there being a treatment that is sitting on shelves because nobody can work out how to pay for it.

Mother of a child with Duchenne Muscular Dystrophy

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The fourth hurdle

A disappointing start...

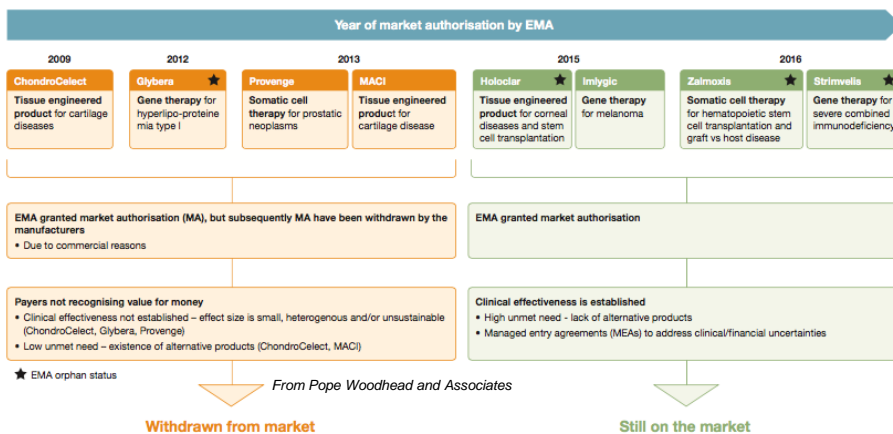


Figure 1: ATMPs approved by the EMA

HTA reviews in Europe

TABLE 8. HTA review

	France	Germany	UK	Italy	Spain	Sweden
Olysetra [†]	Not recommended [†]	Non-quantifiable added benefit [†]	NA	NA	NA	NA
Imlygin [†]	NA	No added benefit	Recommended with restriction	List C/n, not reimbursed	NA	NA
Strimvelis [†]	NA	NA	Recommended	List H	NA	NA
Kymriah [†]	Recommended	Non-quantifiable added benefit	Funded via CDF with CBS scheme	NA	NA	**
Yescarta [†]	Recommended	Non-quantifiable added benefit	Funded via CDF with CBS scheme	NA	NA	***
Luxturna [†]	Recommended	Ongoing G-BA assessment	Ongoing NICE HST assessment	NA	NA	NA
Prowings [†]	NA	Non-quantifiable added benefit [†]	Not recommended [†]	NA	NA	NA
Zalviso [†]	Not recommended	Non-quantifiable added benefit	NA	List H	NA	NA
Aduhelm [†]	Recommended	Non-quantifiable added benefit [†]	Not recommended	NA	NA	NA
Chondroact [†]	Not recommended [†]	Not eligible to have benefit assessment	Recommended	NA	Recommended	NA
MAC2 [†]	NA	Not eligible to have benefit assessment	Recommended with restriction	NA	NA	NA
Holoclax [†]	Recommended with restriction	Non-quantifiable added benefit	Recommended with restriction	List H	NA	NA
Sphero [†]	NA	NA	Recommended	NA	NA	-

NA: Not assessed or no published decision; CDF: cancer drugs fund; CBS: coverage with evidence development; HST: Highly Specialized Technology; ACI: autologous chondrocyte implantation; ALI: Acute lymphoblastic leukaemia; C/n: not yet assessed; N: not; Hospital only, UK: United Kingdom
[†]Decision no more effective as product withdrawn from the market
^{**}Kymriah was assessed by TLV in Sweden. TLV concluded that benefits are associated to high uncertainty and follow-up should be carried out continuously. TLV advice will be considered by county councils for decision making.
^{***}Yescarta was assessed by TLV in Sweden. TLV concluded that benefits are associated to high uncertainty and follow-up should be carried out continuously. TLV advice will be considered by county councils for decision making.
^{****}Luxturna was assessed by TLV in Sweden. TLV concluded that benefits are associated to high uncertainty and follow-up should be carried out continuously. TLV advice will be considered by county councils for decision making.
^{*****}Aduhelm was assessed by TLV in Sweden. TLV concluded that benefits especially in the long term are associated to very high uncertainty. TLV advice will be considered by county councils for decision making.

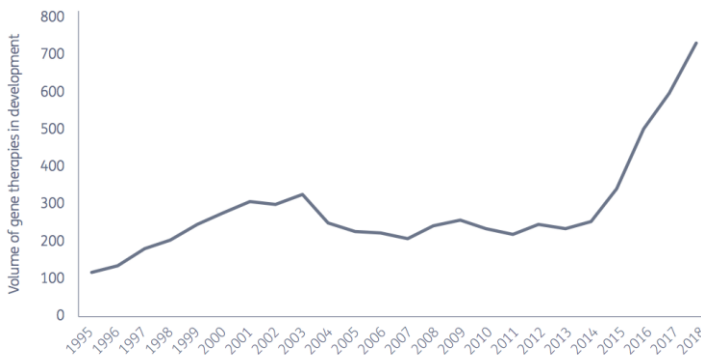
Many European HTA agencies have not reviewed or been unable to approve gene and cell therapies

Germany: mostly “non-quantifiable benefit” which has a significant impact on price

UK: NICE has reviewed 11 of 13 products with most recommended. Luxturna draft guidance issued September 2019 – recommended.

A promising future?

Figure 1. Gene therapy pipeline volume, preclinical through pre-registration phase, 1995–2018



Note: Annual volume snapshots are captured in May of each year.

Source: Pharmaprojects®, August 2018

What would real access look like?

Safe and effective treatments

- Risk
- Benefits - including what is important to patients!

Timely

- Only 40% of NICE HST guidance has been produced within 1 year of marketing authorisation with **30% taking over 100 weeks**

Sustainable (affordable)

- Co-payments
- Health care system

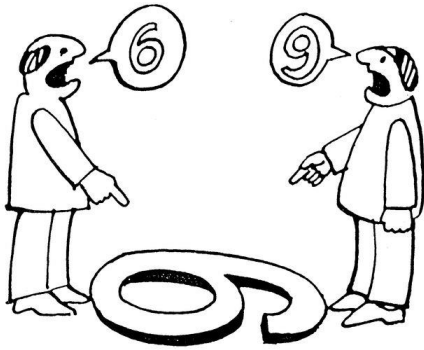
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Patient involvement



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What can patients add?



“Without the patient’s voice, it’s easier to be a little bit more dismissive if you’re looking at clinical data... rather than hearing what effect it had on the individual patient.”

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Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Following the panel: Do you agree we should move away from the application of conventional HTA (at least the cost effectiveness part) for gene therapies?

Live Content Slide

When playing as a slideshow, this slide will display live content

Pre/Post Comparison: Do you agree we should move away from the application of conventional HTA (at least the cost effectiveness part) for gene therapies?