FOUR GENE THERAPIES

DISCLAIMER

- Views and opinions expressed are personal and not necessarily those of UK government, NHS England, Public Health England or other agencies

General issues

- Big payments for one-off treatments
- Payer may not benefit (change of insurer)
- Spread payments
  - For cash flow (not needed?)
  - For assurance of ongoing benefit?

Four Gene Therapies

- Alipogene tipoparvec (Glybera)
- ADA SCID gene therapy (Strimvelis)
- Gene therapy for haemophilia
- Voretigene niparvovec (for Leber congenital amaurosis)

Strimvelis for ADA SCID

- THE DISEASE

- SCID – severe combined immune deficiency
- Many sub types
- ADA – adenosine deaminase (enzyme) deficiency

- Untreated = multiple infections from infancy

- Standard treatment – stem cell transplant (HSCT)
- HSCT is a cure but risk of death during treatment

Severe combined immune deficieny

Strimvelis for ADA SCID

- THE TREATMENT
- Patient's own blood cells infected with gene-carrying virus
- Patient travels to Milan
- Only for patients unsuitable for stem cell transplant (no donor)
- THE COMPARATOR
- Enzyme replacement? (PEG-ADA, Adagen)

Strimvelis for ADA SCID

- THE ECONOMICS

- Priced at 2x enzyme replacement – 594 000 Euro
- ‘75% needed no further enzyme’

- UK comparator?

- Discounting

‘… for life’

Time horizon for the analysis

Discounting

Voretigene niparvovec

- THE DISEASE
- Leber’s congenital amaurosis
- Blindness from childhood
- RPE 65 gene
- 19 known genes; 30% of patients no known mutation
- 1 in 50 000 births

Voretigene niparvovec

- THE TREATMENT
- Gene therapy injected directly into the eye
- Improves ability to navigate an obstacle course at 1 lux
- Patients aged 4 yrs to middle age
- THE COMPARATOR

Voretigene niparvovec

- THE ECONOMICS
- QALY gain
- One eye or two?
- Old or young?
- 10 new patients per annum

Alipogene tipoparvec (Glybera)

- THE DISEASE
- LPLD – lipoprotein lipase deficiency
- Attacks of pancreatitis – very painful, can be fatal

Alipogene tipoparvec

- THE TREATMENT
- Gene therapy injected into muscle
- THE ECONOMICS
- Priced at $1m per patient

Biomedicine

The World's Most Expensive Medicine Is a Bust

The first gene therapy approved in the Western world costs $1 million and has been used just once. The doctor who tried it says the price is "absolutely too high."

by Antonio Regalado May 4, 2016

BMN 270 FOR HAEMOPHILIA

THE DISEASE

Clotting disorder – bleeding into joints or brain

BMN 270

THE TREATMENT

Gene therapy

Mean annualised infusions of FVIII fell from 137 to 3

Based on 6 out of 7 patients who received ‘high dose’ therapy

Follow up of 34 – 50 weeks

THE COMPARATOR

Factor VIII replacement

Natural or recombinant?

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THE ECONOMICS

6 000 people in England with haemophilia

£230m spend = £400k per patient