EURORDIS SYNDROME – A SCENARIO
Payer/ HTA Position – not for public view

- Stagnant, financially constrained budget
- Other priorities that will have an impact on public health issues for a wider population
- Current treatment option supports symptom management
- If I agree this, it will set a precedence for others
- The health system needs to be sustainable
- Other more prevalent conditions will need to be sacrificed to fund this
Patient/parent perspective - not for public view

- Lost one child to EURORDIS Syndrome
- Second child with mild physical impairments; has received Genordis in clinical trial
- Need to move home and pay for modifications to the house for accessibility purposes
- Giving up work to care for their child full time
- High out of pocket expenses for travel to medical appointments
- Payments for additional help such as physiotherapy
Financially viable

Genordis very efficacious and perceived as a cure

Company commits to future investment to improve on Genordis: earlier diagnosis and prevention

Return on investment needed (rare disease, e.g. small market): EUR 456mio revenue is a minimum to fund production facility, clinical trials and patient support

US finances EU and global research

Company anticipates cost saving for the wider health and social care system

Company mentions lack of trustworthiness of the payer: fail to have clarity & certainty on the longer term budget for Genordis