



EURODIS 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

Pharmaceutical perspective - not for public view

- ▶ 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