Patients and Families Priorities – Gaucher Disease

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Gaucher Disease

- A short questionnaire devised by the EGA sent to all our member countries - 29/6 outside Europe
- Parents of children, not young people
Patient Views

• Questionnaire sent by the EGA to Type I patients/families to identify their position on clinical trials – 2011
• Feedback from EGA member countries on access to different ERTs
• Age range 7 to 16 years
EGA Paediatric patient numbers are:
Austria – 2
Bulgaria – 2
Czech Republic – 12
Denmark – 4
Finland – 2
France – 80 - % of children unknown
Germany – 40
Greece – 10
Italy – 13
Latvia – 1
Netherlands – 5-7
Norway – 15
Poland - 15
Romania – 8
Slovenia – 2
Spain – 100
Sweden – 10
UK – 33
**Total ~ 160-200**
Different ERTs

• Choice of ERT
  – Availability to ERT is limited in many countries i.e. Elelyso not available in Europe, VPRIV not approved yet and not licensed for Type III patients
  – Clinical decision based on experience; availability; individual patient issues.
  – For every new patient the clinician alternates the ERT for equity.
    • Parents do have a good knowledge through their work of the patient associations of the different products
What are the unmet clinical needs from a patients’ point of view?

- Fatigue
- Bone problems
- Growth problems
- Neurological aspects
Clinical Trials - Opportunities

- Unmet need
- Shorter infusion time
- Different type of drug – oral
- Less frequent administration
Clinical Trials – Challenges (Type I only)

- Small numbers
- Clinical Fatigue
- Travel & Cost
- Number of hospital visit & tests
- New patients – more severe of challenging countries
- Switch - incentives
Clinical Trials – Challenges (Type III only)

- The right end points, trial design needs to be improved to accommodate young children
- Pharmaceutical companies need to understand the importance of liquid formulations
- “Trial fatigue”
- Small number of patients
- All therapies may not suit all patients; this may divide families
- Mouse model but no large animal model so many unanswered questions
Thank You