



EGA

European Gaucher Alliance

Driven by passion for patients

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. Elclyso 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 or 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