Ways to Measure Clinical Effectiveness in the Investigation of Medicinal Products for the Treatment of DMD/BMD
Friday 21st June 2013

What is clinically meaningful and how to measure this?
Duchenne and Becker muscular dystrophy

- Hereditary, X-linked diseases
- Complete (DMD) or partial loss (BMD) of dystrophin
- **Severe and progressive disease:**
  - Skeletal muscle weakness
  - Respiratory insufficiency
  - Cardiomyopathy
  - Behavioral and cognitive problems (1/3 of DMD)
- New and recent data on *major disease milestones*
- *Clinical meaningful* changes for all stages of the disease
- *Outcome measures* for all different age groups
Conclusions Session 1

• New standards of care, ventilatory support, and corticosteroid treatment have led to improvement in survival and major disease milestones.

• *Clinical meaningful* changes are possible at all stages of the disease.

• *New data* show that changes in one disease milestone predict changes of other major milestones.

• *Outcome measures* are available for the ambulatory patients.

• *Outcome measures* are in development for younger children (3-5 years) and non-ambulant boys.