Muscle wasting and weakness are among the most common symptoms of the muscular dystrophies, cachexia, and age-related wasting diseases. Consequently, there is considerable interest in finding ways to improve muscle bulk and strength.

**Myostatin-based Therapies**

**Myostatin**: A growth factor that negatively regulates skeletal muscle mass

Mutations in myostatin are associated with gross muscle mass seen in several species of cattle, dogs, and mice.

A natural myostatin mutation has been identified in a young boy.

Several strategies are being developed to knockdown its expression to improve muscle-wasting conditions.

- **Exon-skipping approach** (see Exon skipping poster)
  Myostatin knockdown by exon-skipping leads to increased muscle mass.

- **Pro-peptide approach**
  Myostatin is inhibited by its own pro-peptide (a precursor form of the protein)
  Treatment of dystrophic mice with myostatin pro-peptide leads to an increase in skeletal muscle mass.

These therapies are being researched in several muscle wasting disease models including OPMD & DMD.

**What is OPMD?**

**Cause** - Oculopharyngeal muscular dystrophy (OPMD) is caused by an abnormal gene (PABPN1) and its resulting protein aggregation in muscle cells.

**Onset** - mid 40s to 50s

**Symptoms** - weakness of the muscles of the eyelids and throat. Weakness of facial and limb muscles often occurs later in disease, leading to swallowing problems and difficulty keeping the eyes open.

**Incidence** - 1 in 100,000 in European population

**Treatments** - no specific treatment
  Physiotherapy for muscle weakness & corrective eyelid surgery