**Pulmonary Function Is Stable Through Week 144 in Patients With Duchenne Muscular Dystrophy (DMD)**

**INTRODUCTION**

**Duchenne Muscular Dystrophy (DMD): Devastating Rare Disease**
- Rare X-linked recessive muscular dystrophy
- BDM is caused by mutations in the gene for dystrophin, a protein that plays a key role in cell and muscle function.

**Exon-Skipping Drug: Eteplirsen**
- Addresses the underlying cause of DMD
- Eteplirsen is a morpholino-oligonucleotide that targets alternative splicing.

**PMO restores dystrophin in diaphragm: MDX mouse model**
- Eteplirsen restores dystrophin expression in diaphragm muscle of transgenic mdx mice.

**Pulmonary Function Tests: Importance for DMD**
- Measures the total volume of air expelled during forced exhalation after maximum inspiration.
- MIP, MEP, and FVC are primary and secondary endpoints in DMD clinical trials.

**METHODS + RESULTS**

**Eteplirsen Phase III Studies**
- Randomized, double-blind, placebo-controlled trials.

**Patient Characteristics at Baseline**
- Table 1: Demographics and baseline characteristics.

**Summary of Pulmonary Function Tests: Week 144 Treatment Results in ITT Population (n=12)**
- Table 2: Summary of pulmonary function tests.

**CONCLUSIONS**
- Eteplirsen demonstrated stability on PFTs in the ITT population (n=12), as measured by MIP, MEP, FVC, MIP % predicted, MEP % predicted and FVC % predicted from baseline through Week 144.
- The reported stability on PFT measurements over nearly 5 years is contrary to a steady decline observed in the DMD natural history.