Q&A on A083-02: the Phase 2 ACE-083 Clinical Study in FSHD

1. **What is the A083-02 study?**
   This study is designed to evaluate the safety, tolerability and efficacy of ACE-083 in up to 76 adult patients with facioscapulohumeral muscular dystrophy (FSHD). This study will occur in two parts: Part 1 will be used to assess the safety, tolerability and the optimal dose of ACE-083 in patients with FSHD; Part 2 will be used to determine whether the muscle volume, strength, and function are increased when compared to treatment with a placebo. Placebo is an inactive substance that looks the same as and is given in the same way as ACE-083. Importantly, everyone who takes part in the ACE-083 study will receive standard of care treatment and be closely monitored by the study staff.

   Part 1 of the study is planned to take place at multiple sites in the United States. Part 2 is planned to involve sites in the United States, Canada and the European Union. Total study duration for each patient will be approximately 24 weeks, including a 4-week screening period, a 12-week treatment period, and an 8-week follow-up period after the last dose.

2. **What is ACE-083 and what does it do?**
   ACE-083 is an investigational drug that inhibits selected proteins in the transforming growth factor-beta (TGF-β) superfamily involved in the regulation of muscle mass and strength. ACE-083 has been designed to increase muscle mass and strength selectively in the muscles into which the drug is injected. Acceleron is developing ACE-083 for diseases in which improved muscle strength in a specific set of muscles may provide a clinical benefit to patients, such as FSHD. As an "investigational" drug, ACE-083 is not approved by any regulatory agency for use in any country.

3. **Who should consider participating in the A083-02 study?**
   Patients who are 18 years of age or older and have genetically confirmed FSHD or patients with a clinical diagnosis of FSHD and a first-degree relative with genetically confirmed FSHD may be eligible to take part in the ACE-083 study. Eligible patients must also be experiencing mild to moderate weakness in their tibialis anterior and/or biceps brachii muscles. Several additional criteria will be considered. Your doctor is the best source of information about your potential treatment options. If you have questions about taking part in this clinical study, ask your doctor for more information.

4. **Where can I find out more information?**
   When considering participation in a clinical study, it is best to speak with your physician. You may also learn more about the ACE-083 study in FSHD by visiting www.clinicaltrials.gov and searching for study identifier NCT02927080 or by emailing clinicaltrials083@acceleronpharma.com.