Monday February 24th, 2020,

On Monday, February 24th, 2020, the National Institute of Health’s clinical trials database, clinicaltrials.gov was updated with information regarding Pfizer’s Phase 3, randomized, double-blind, placebo-controlled study of PF-06939926 (mini-dystrophin gene therapy), exploring the safety and efficacy of gene therapy in boys with Duchenne muscular dystrophy (DMD).

This is an important first step in informing researchers, clinicians and families about our intent to initiate the next clinical study in our gene therapy clinical development program by late spring or early summer. The exact timeline is still evolving. Specific details about the participating countries and sites will be announced on clinicaltrials.gov and shared with the community as they are finalized.

Key inclusion criteria:
- Boys aged 4 to 7 years “up to their 8th birthday” (i.e., age ≥4 and <8 years) at screening
- Confirmed diagnosis of DMD by prior certified clinical genetic testing
- Treated with stable, daily dose of glucocorticoids for ≥3 months at screening
  - ≥0.5 mg/kg prednisone or prednisolone, or ≥0.75 mg/kg deflazacort
- Ambulatory as assessed by protocol-specified criteria

Key exclusion criteria:
- Positive test performed by Pfizer for neutralizing antibodies to AAV9
- Any treatment designed to increase dystrophin expression within 6 months prior to screening
  - Examples: Translarna™, EXONDYS 51™, VYONDYS 53™
- Any prior treatment with gene therapy
- Any injury that may impact functional testing
  - Leg fractures must have occurred >3 months prior to screening and be fully healed at screening
- Abnormality in specified laboratory tests, including blood counts, liver, and kidney function

More information will become available as we progress. Throughout this program, we have collaborated with DMD patients, parents, advocates, researchers and clinicians to inform our clinical trial approach. Collaboration is an important aspect of our program and we want to express our gratitude to all who have guided us. We will continue to partner with patient organizations and the broader DMD community, to ensure that information about the trial is shared widely.