

August 12, 2020

Dear DMD Community,

On behalf of NS Pharma, I am delighted to share that VILTEPSO™ (viltolarsen) injection received an Accelerated Approval by the FDA today based on increases in dystrophin, for the treatment of Duchenne muscular dystrophy (DMD) in patients amenable to exon 53 skipping therapy. In a clinical trial of 16 patients, aged four to less than 10 years:

- Patients who received the recommended dose of 80 mg/kg/wk (N=8) showed an increase in dystrophin expression to an average of 5.9% of normal after about 5-6 months of treatment versus 0.6% at baseline.
- Overall, 100% of patients (8/8) treated with VILTEPSO 80 mg/kg/wk showed an increase in dystrophin levels after treatment and 88% of patients (7/8) showed dystrophin levels of 3% of normal or greater.
- The most common adverse reactions in patients treated with VILTEPSO included upper respiratory tract infection, injection site reaction, cough and fever.

VILTEPSO is also the first and only exon 53 skipping therapy to demonstrate an increase in dystrophin in children as young as four years old. Patients receiving treatment with VILTEPSO have the option and flexibility to receive infusions at their home or at a hospital or treatment center. VILTEPSO is administered by a trained healthcare professional as an 80 mg per kg of body weight 60-minute weekly intravenous infusion. For additional information, please see the full VILTEPSO [Prescribing Information](#).

We encourage you to share this news and hope you will join us for a series of webinars on the individualized resources and comprehensive care coordination available through NS Support. You can follow us on [LinkedIn](#) and [Twitter](#) for information and registration for upcoming webinars.

This Accelerated Approval has been a milestone many years in the making – and it could not have been achieved without the unflagging support of the DMD community. On a daily basis we are inspired by the parents, children, doctors, nurses and other healthcare professionals enthusiastically working towards a brighter future.

We are deeply thankful for everything you do and excited about making a difference in DMD, together.

Thank you,
Tsugio Tanaka
President,
NS Pharma, Inc.

Indication

VILTEPSO is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VILTEPSO. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

IMPORTANT SAFETY INFORMATION

- In clinical studies, no patients experienced kidney toxicity during treatment with VILTEPSO. However, kidney toxicity from drugs like VILTEPSO may be possible. Your doctor may monitor the health of your kidneys before starting and during treatment with VILTEPSO.
- The most common side effects of VILTEPSO included upper respiratory tract infection, injection site reaction, cough and fever.

For additional safety information, please see the full [Prescribing Information](#).