140 East Ridgewood Avenue, Suite 280S
Paramus, NJ 07652, USA

NS Pharma Community Letter

Dear Friends,

NS Pharma is pleased to announce that we have initiated submission of a rolling New Drug Application (NDA) seeking accelerated approval for viltolarsen (NS-065/NCNP-01), an investigational drug for the treatment of Duchenne Muscular Dystrophy (DMD) in patients amenable to exon 53 skipping. While this is the first step towards the US regulatory approval, we wanted to share our news with the DMD community.

As you may know, NS Pharma has completed a Phase 2 study in North America, and its parent company, Nippon Shinyaku Co., Ltd. (Headquarters, Kyoto, Japan; President, Shigenobu Maekawa) completed a Phase 1/2 study in Japan. The scientists, investigators, and families all made great efforts to collect data about the safety and efficacy of viltolarsen (NS-065/NCNP-01) in clinical trials. Viltolarsen (NS-065/NCNP-01) is not approved in any country for use outside clinical trials.

Working together to analyze the data, we have decided to move forward to seek regulatory approval. We have submitted the first portion of the data and reports to the US Food and Drug Administration (FDA) under rolling review pathway. Under that pathway, the sponsor is allowed to start submission with completed portions of the application for review by FDA rather than waiting until every portion is completed. NS Pharma plans to complete the NDA submission in September 2019.

Finally, we would like to take this moment to thank all the researchers, clinical trial staff, and families around the world who have made this step possible. We deeply appreciate everyone's hard work and dedication, and thank you for your continued support.

Thank you,

Tsugio Tanaka

President, NS Pharma, Inc.

For family or community questions: Email dmdresearch@nspharma.com

To learn more about viltolarsen (NS -065/NCNP-01): Please visit www.nspharma.com