

Summary of Investigation Results

Viltolarsen

Non-proprietary name	Non-proprietary name	Brand name (marketing authorization holder)
Brand name	Viltolarsen	Viltepso Injection 250 mg (Nippon Shinyaku Co., Ltd.)
Indications	Duchenne muscular dystrophy with a deletion in the dystrophin gene amenable to exon 53 skipping therapy	
Summary of revisions	1. In the section "5. PRECAUTIONS CONCERNING INDICATIONS", precautions were revised to state that carefully decide whether to start and continue administration after thoroughly understanding the efficacy and safety of this drug and be thoroughly familiar with the contents of "CLINICAL STUDIES" section. In addition, the patient's condition should be carefully monitored after administration and the efficacy should be evaluated periodically to determine whether to continue administration. If no effect is observed, administration should be discontinued. 2. In the section "11.2 Other Adverse Reactions," the adverse events and the incidence of adverse reactions should be updated based on adverse reactions observed in the multinational phase III study (NS-065/NCNP-01-301) and other studies. 3. The results of the multinational phase III study (NS-065/NCNP-01-301) should be added to "17. CLINICAL STUDIES" section.	
Investigation results and background of the revision	As a result of deliberations that the results from the multinational phase III study (NS-065/NCNP-01-301) conducted as a post-marketing clinical study were submitted, following a result of a review based on the opinions of the expert advisors, and given the need to provide appropriate information to the medical community, MHLW/PMDA concluded that revision of PRECAUTIONS was necessary.	

The expert advisors present at the Expert Discussion regarding the current investigation were nominated based on their conflict of interest declarations concerning the relevant products, pursuant to the "Rules for Convening Expert Discussions, etc., by the Pharmaceuticals and Medical Devices Agency" (PMDA Administrative Rule No. 20-8, dated December 25, 2008).