Summary of Investigation Results

Aceneuramic acid

November 13, 2024

Non-proprietary name

Aceneuramic acid

Brand name (marketing authorization holder)

Acenobel Extended Release Tablets 500 mg (Nobelpharma Co., Ltd.)

Japanese market launch

Unreleased

Indications

Delaying the progression of muscle weakness in patients with distal myopathy with rimmed vacuoles

Summary of revisions

Precautions for impurities for which genotoxicity has not been evaluated should be deleted in the 8. IMPORTANT PRECAUTIONS section and the 15.2 Information Based on Nonclinical Studies section in 15. OTHER PRECAUTIONS.

Investigation results and background of the revision

Among impurities above the qualification threshold, for which safety confirmation is required as specified in the ICH Q3A Guideline (“Revision of IMPURITIES IN NEW DRUG SUBSTANCES” (PMSB/ELD Notification No.1216001 dated December 16, 2002), the results of genotoxicity studies were evaluated for 3 types of impurities for which genotoxicity had not been evaluated. As a result of consultation with expert advisors, since no special concerns were raised in the relevant studies, the MHLW/PMDA concluded that revision of

PRECAUTIONS was appropriate.

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).