

# New quality control method to select effective M-beta-CD for treating Neimann-Pick disease

September 20 2017

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Researchers have developed a quality control method to evaluate the pharmacological activity and potential effectiveness of different preparations of the therapeutic agent methyl- $\beta$ -cyclodextrin (M $\beta$ CD). Distinct batches of M $\beta$ CD produced by different commercial laboratories may be more or less effective in reducing the cholesterol that accumulates in the fibroblasts of patients with the lysosomal storage disorder Niemann-Pick disease type C1. This new set of methods for selecting an optimal cyclodextrin preparation is described in *ASSAY and Drug Development Technologies*.

Wei Zheng, PhD, National Center for Advancing Translational Sciences, National Institutes of Health (Bethesda, MD) and coauthors from the NIH, Poochon Scientific (Frederick, MD), and Washington University School of Medicine (St. Louis, MO), developed an analytical method that they used to characterize three preparations of M $\beta$ CD. The researchers found differences in average molecular weight and side chain methylation in M $\beta$ CD samples from different vendors and batches. They discuss the potential impact of these differences on the ability of M $\beta$ CD to reduce lysosomal cholesterol accumulation in the article entitled "Analytical Characterization of Methyl- $\beta$ -Cyclodextrin for Pharmacological Activity to Reduce Lysosomal Cholesterol Accumulation in Niemann-Pick Disease Type C1 Cells."

"This scientific disclosure highlights the importance of consistency in batch production of M $\beta$ CD. Without this technology, there would be little promise to develop a clinical treatment for these patients, many of whom are young children," says *ASSAY and Drug Development Technologies* Editor-in-Chief Bruce Melancon, PhD, Managing Director of the Chemical Synthesis and Drug Discovery facility at the University of Notre Dame.

**More information:** Rong Li et al, Analytical Characterization of Methyl- $\beta$ -Cyclodextrin for Pharmacological Activity to Reduce Lysosomal Cholesterol Accumulation in Niemann-Pick Disease Type C1 Cells, *ASSAY and Drug Development Technologies* (2017). [DOI: 10.1089/adt.2017.774](https://doi.org/10.1089/adt.2017.774)

Provided by Mary Ann Liebert, Inc

Citation: New quality control method to select effective M-beta-CD for treating Neimann-Pick disease (2017, September 20) retrieved 3 May 2024 from <https://medicalxpress.com/news/2017-09-quality-method-effective-m-beta-cd-neimann-pick.html>

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