Catalog # 10-2413

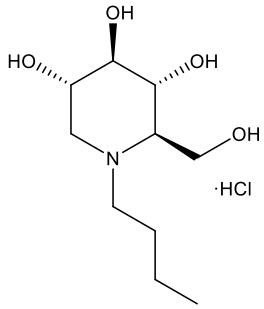
Miglustat HCl

CAS# 210110-90-0

N-Butyldeoxynojirimycin HCl

NB-DNJ

Lot # X101478



Orally active ceramide-specific glycosyltransferase and α-glucosidase I and II inhibitor.[1] Rescues trafficking-deficient F508del-CFTR in human airway epithelial cells via inhibition of ER α-glucosidases I and II.[2] Pharmacological chaperone for glucocerebrosidase degradation.[3] Clinically useful agent for Gaucher disease type 1.[4] Stabilizes neurological disorders in Niemann-Pick disease type C.[5]

1) Platt et al. (1994), *N-butyldeoxynojirimycin is a novel inhibitor if glycolipid biosynthesis*; J. Biol. Chem., **269** 8362
2) Noel *et al*. (2008), *Parallel improvement of sodium and chloride transport defects by miglustat (n-butyldeoxynojyrimicin) in cystic fibrosis*; J. Pharmacol. Exp. Ther., **325** 1016
3) Abian *et al*. (2011), *Therapeutic strategies for Gaucher disease: miglustat (NB-DNJ) as a pharmacological chaperone for glucocerebrosidase and the different thermostability of velaglucerase alfa and imiglucerase*; Mol. Pharm., **8** 2390
4) Serratrice *et al*. (2015), *Switching from imiglucerase to miglustat for the treatment of French patients with Gaucher disease type 1: a case series*; J. Med. Case Rep., **9** 146
5) Karimzadeh (2013), *Effects of miglustat on stabilization of neurological disorder in niemann-pick disease type C: Iranian pediatric case series*; J. Child Neurol., **28** 1599

PHYSICAL DATA

Molecular Weight:	255.74
Molecular Formula:	$C_{10}H_{21}NO_4$ HCl
Purity:	98% by TLC
	NMR: (Conforms)
Solubility:	Soluble in DMSO (up to 20 mg/ml) or in water (up to 20 mg/ml)
Physical Description:	White solid
Storage and Stability:	Store as supplied desiccated at -20°C for up to 1 year from the date of purchase.
	Solutions in DMSO or distilled water may be stored at -20°C for up to 1 month.

Focus Biomolecules LLC 400 Davis Drive, Suite 600 Plymouth Meeting PA 19462

www.focusbiomolecules.com