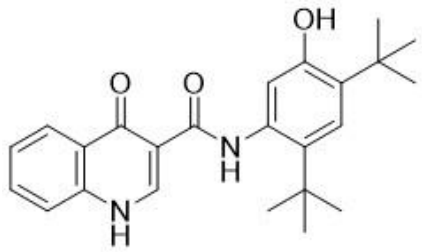
Product data sheet

MedKoo Cat#: 507210 Name: Ivacaftor CAS#: 873054-44-5 Chemical Formula: $C_{24}H_{28}N_2O_3$ Exact Mass: 392.20999 Molecular Weight: 392.49072	
Product supplied as:	Powder
Purity (by HPLC):	≥ 98%
Shipping conditions	Ambient temperature
Storage conditions:	Powder: -20°C 3 years; 4°C 2 years.
	In solvent: -80°C 3 months; -20°C 2 weeks.

1. Product description:
Ivacaftor, also known as VX-770, is a drug used to treat cystic fibrosis in people with certain mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene, who account for 4–5% cases of cystic fibrosis, and is included in a combination drug, lumacaftor/ivacaftor, which is used to treat people with cystic fibrosis who have the F508del mutation in CFTR. Ivacaftor is a "potentiator" of CFTR, meaning it increases the probability that the defective channel will be open and allow chloride ions pass through the channel pore.

2. CoA, QC data, SDS, and handling instruction
SDS and handling instruction, CoA with copies of QC data (NMR, HPLC and MS analytical spectra) can be downloaded from the product web page under "QC And Documents" section. Note: copies of analytical spectra may not be available if the product is being supplied by MedKoo partners. Whether the product was made by MedKoo or provided by its partners, the quality is 100% guaranteed.

3. Solubility data

Solvent	Max Conc. mg/mL	Max Conc. mM
DMSO	50.0	127.39

4. Stock solution preparation table:

Concentration / Solvent Volume / Mass	1 mg	5 mg	10 mg
1 mM	2.55 mL	12.74 mL	25.48 mL
5 mM	0.51 mL	2.55 mL	5.10 mL
10 mM	0.25 mL	1.27 mL	2.55 mL
50 mM	0.05 mL	0.25 mL	0.51 mL

5. Molarity Calculator, Reconstitution Calculator, Dilution Calculator
Please refer the product web page under section of "Calculator"

6. Recommended literature which reported protocols for in vitro and in vivo study
In vitro study

1. Van Goor F, Yu H, Burton B, Hoffman BJ. Effect of ivacaftor on CFTR forms with missense mutations associated with defects in protein processing or function. J Cyst Fibros. 2014 Jan;13(1):29-36. doi: 10.1016/j.jcf.2013.06.008. Epub 2013 Jul 23. PMID: 23891399.
2. Yu H, Burton B, Huang CJ, Worley J, Cao D, Johnson JP Jr, Urrutia A, Joubran J, Seepersaud S, Sussky K, Hoffman BJ, Van Goor F. Ivacaftor potentiation of multiple CFTR channels with gating mutations. J Cyst Fibros. 2012 May;11(3):237-45. doi: 10.1016/j.jcf.2011.12.005. Epub 2012 Jan 30. PMID: 22293084.

In vivo study
TBD

7. Bioactivity
Biological target:
Ivacaftor (VX-770) is a potent CFTR potentiator, targeting G551D-CFTR and F508del-CFTR with EC50s of 100 nM and 25 nM, respectively.

Product data sheet

In vitro activity

The aim of this in vitro study was to evaluate the effect of ivacaftor on mutant CFTR protein forms with defects in protein processing and/or channel function. The effect of ivacaftor on CFTR function was tested in electrophysiological studies using a panel of Fischer rat thyroid (FRT) cells expressing 54 missense CFTR mutations that cause defects in the amount or function of CFTR at the cell surface. Acute (5-min) addition of ivacaftor following CFTR activation by forskolin significantly (P = 0.05; paired t-test) increased chloride transport over baseline for a number of the mutant CFTR forms tested (Table 2; Fig. 2B). The net increase over baseline chloride transport by ivacaftor (ivacaftor response minus baseline) ranged from 1.8 to 155.0% of normal CFTR, reaching maximum sustained levels of 2.1 to 200.7% of normal CFTR (Table 2; Fig. 2B). The fold increase over baseline chloride transport (ivacaftor response divided by baseline) ranged from 1.6 to 52.0 (Table 2). The EC50 of ivacaftor for all mutant CFTR forms tested was similar to G551D-CFTR (range; 101 to 735 nM) (Table 2; Fig. 3). The remaining mutant CFTR forms had no significant response to ivacaftor under the experimental conditions used in this study (Table 2; Fig. 2B). In conclusion, ivacaftor potentiated multiple mutant CFTR forms produced by missense CFTR mutations expressed in a panel of FRT cells. These in vitro studies along with in vivo measures of residual CFTR function, such as exocrine pancreatic function or sweat chloride concentrations, could be used to help stratify patients with CF who have different CFTR genotypes for studies investigating the potential clinical benefit of ivacaftor.

Reference: J Cyst Fibros. 2014 Jan;13(1):29-36. https://pubmed.ncbi.nlm.nih.gov/23891399/

In vivo activity

TBD

Note: The information listed here was extracted from literature. MedKoo has not independently retested and confirmed the accuracy of these methods. Customer should use it just for a reference only.