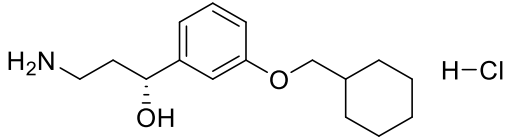


# Product data sheet



MedKoo Cat#: 319694 Name: Emixustat HCl CAS#: 1141934-97-5 (HCl salt) Chemical Formula: C <sub>16</sub> H <sub>26</sub> ClNO <sub>2</sub> Molecular Weight: 299.839	
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:

Emixustat, also known as ACU-4429, is a visual cycle modulator in development for the potential treatment of GA associated with dry AMD. Emixustat is a nonretinoid compound with a unique mode of action in the retinal pigment epithelium, where it modulates the biosynthesis of visual chromophore through its effect on retinal pigment epithelium-specific 65 kDa protein isomerase.

## 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	44.0	146.75

## 4. Stock solution preparation table:

Concentration / Solvent Volume / Mass	1 mg	5 mg	10 mg
1 mM	3.34 mL	16.68 mL	33.35 mL
5 mM	0.67 mL	3.34 mL	6.67 mL
10 mM	0.33 mL	1.67 mL	3.34 mL
50 mM	0.07 mL	0.33 mL	0.67 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. Bavik C, Henry SH, Zhang Y, Mitts K, McGinn T, Budzynski E, Pashko A, Lieu KL, Zhong S, Blumberg B, Kuksa V, Orme M, Scott I, Fawzi A, Kubota R. Visual Cycle Modulation as an Approach toward Preservation of Retinal Integrity. PLoS One. 2015 May 13;10(5):e0124940. doi: 10.1371/journal.pone.0124940. PMID: 25970164; PMCID: PMC4430241.

### In vivo study

1. Kubota R, Calkins DJ, Henry SH, Linsenmeier RA. Emixustat Reduces Metabolic Demand of Dark Activity in the Retina. Invest Ophthalmol Vis Sci. 2019 Nov 1;60(14):4924-4930. doi: 10.1167/iovs.19-28194. PMID: 31770432.  
2. Zhang J, Kiser PD, Badiie M, Palczewska G, Dong Z, Golczak M, Tochtrop GP, Palczewski K. Molecular pharmacodynamics of emixustat in protection against retinal degeneration. J Clin Invest. 2015 Jul 1;125(7):2781-94. doi: 10.1172/JCI80950. Epub 2015 Jun 15. PMID: 26075817; PMCID: PMC4563688.

## 7. Bioactivity

### Biological target:

Emixustat hydrochloride strongly inhibits 11-cis-retinol production with IC<sub>50</sub> values of 232 ± 3 nM.

### In vitro activity

# Product data sheet



The production 11-cis-retinol (11-cis-ROL) was measured by HPLC (Fig 2A). The data showed a concentration-dependent reduction of 11-cis-ROL production (i.e., inhibition of RPE65 isomerase activity) by emixustat.

Reference: PLoS One. 2015; 10(5): e0124940. <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4430241/>

## In vivo activity

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In vehicle-treated animals, cation channel activity increased in the dark. Emixustat treatment reduced cation channel activity; activity was comparable to vehicle-treated controls in light conditions. In vehicle-treated rats, minimum retinal oxygen tension decreased as the retina recovered from a photobleach, indicating that more oxygen was being consumed. Emixustat treatment prevented the decrease in oxygen pressure after photobleach.

Reference: Invest Ophthalmol Vis Sci. 2019 Nov 1;60(14):4924-4930. <https://pubmed.ncbi.nlm.nih.gov/31770432/>

*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.*