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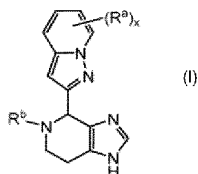
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(54) Title: 4-PYRAZOLO[1,5-A]PYRIDIN-2-YL-4,5,6,7-TETRAHYDRO-1H-IMIDAZO[4,5-C]PYRIDINE DERIVATIVES AS MUTANT PAH PROTEIN STABILIZERS FOR THE TREATMENT OF PHENYLKETONURIA



(57) Abstract: The disclosure is directed to 4-pyrazolo[1,5-a]pyridin-2-yl-4,5,6,7-tetrahydro-1H-imidazo[4,5-c]pyridine derivatives of formula (I) and pharmaceutically acceptable salts thereof, that stabilize phenylalanine hydroxylase (PAH) mutations, pharmaceutical compositions comprising those compounds, and methods of using those compounds in methods for stabilizing a mutant PAH protein or reducing blood phenylalanine concentration in a subject suffering from phenylketonuria. In some embodiments, the mutant PAH protein contains at least one R408W, R261Q, R243Q, Y414C, L48S, A403V, I65T, R241C, L348V, R408Q, or V388M mutation. In other embodiments, the mutant PAH protein contains at least one R408W, Y414C, I65T, F39L, R408Q, L348V, R261Q, A300S, or L48S mutation.

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