Restoring Physiology in Iron-Deficient Organisms with a Small Molecule

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Many genetic diseases result from protein iron-transporter deficiencies, including microcytic anemia, caused by insufficient levels of functional divalent metal transporter 1 (DMT1). A common consequence of this deficiency is a buildup of iron gradients across lipid membranes. We discovered a small molecule that can autonomously transport iron into, within, and out of cells thereby restoring physiology in both cellular and animal models of DMT1 deficiency. Further evidence supports functional collaboration between the small molecule and endogenous proteins involved in iron transport. Collectively, these findings demonstrate the potential of utilizing imperfect small molecule mimics as replacements for missing protein-iron transporters as a general therapeutic strategy for many different human diseases.