Involvement of TorsinA in CFTR Processing: New Insights from a C. elegans Model.

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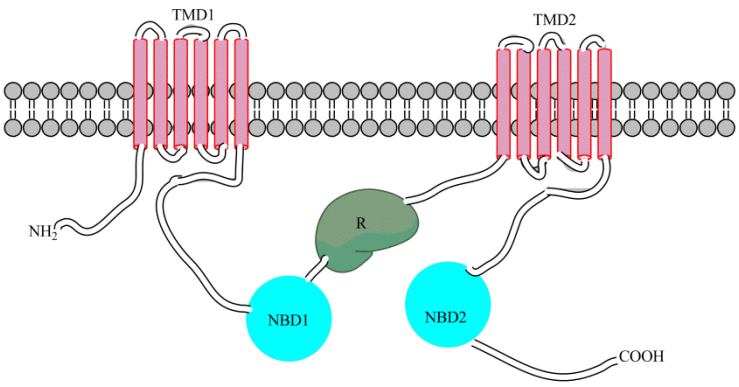

Cystic fibrosis is an inherited disorder caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR). CFTR is an ABC ion transporter that functions as an anion channel at the cell surface of many epithelial cells. The deletion of F508 in CFTR only minimally disrupts protein function but results in the mutant protein being rapidly degraded in the endoplasmic reticulum by ER quality control mechanisms thus causing the disease phenotype. Modulation of quality control mechanisms to prevent CFTR degradation and to promote proper CFTR maturation may ameliorate the disease phenotype in human patients. Recently, torsinA a protein with chaperone functions has been implicated in the turnover of mutant CFTR. We have developed a new CF model in *C. elegans* to both study how torsinA is involved in CFTR turnover as well as uncover other proteins involved in CFTR processing. Furthermore, we have examined chemical modulation of torsinA activity as a potential CF therapeutic.