



STEP UP! TO END AHC



Ella, Age 4

\$500,000 GOAL

NOVEMBER 1 - DECEMBER 31

www.StepUp4AHC.org

4 Projects 4 AHC

Begin new gene therapy project to correct ATP1A3 mutation. An intentional collaborative effort (AAV Project) benefiting from the expertise leading researchers and using each of the below projects.

Test actual AHC neurons to see where the problems are occurring in patients. Northwestern/Vanderbilt universities.

Care for AHC Mouse Colonies at Northwestern University.

Use iPS cells with AHC mutations to test therapeutic strategies at Vanderbilt Univ.

