

Dear Members of the CIRM Board,

RE: Letter of support for CLIN2 17091

We are writing to ask for your support of Elpida's clinical trial for SPG50. What makes this clinical trial so crucial is that unlike many rare diseases, SPG50 slowly robs a child of basic skills we all take for granted – walking, talking, self-care, etc. SPG50 does not stand still. Our niece, Naomi Lockhard, who is diagnosed with SPG50, has worked tirelessly in therapies to make gains and without gene therapy, she will lose all those gains and more. Your support for Elpida's SPG50 clinical trial also goes beyond just SPG50 - investing in this type of gene therapy is an investment in moving the technological needle further in treating many rare conditions. We know funding decisions are difficult, but we are hopeful that you will view supporting Elpida's SPG50 clinical trial as supporting emerging technology that will benefit all rare diseases.

Thank you for your time.

Warm regards,

Paula Cerrone and Scott Stern