We write to you today not just as advocates, but as an aunt and uncle who have experienced both the joy and heartbreak of watching our niece and nephew grow. There is nothing more incredible than seeing a child take their first steps or hearing their laughter as they crawl across the floor. We've celebrated those milestones with both of them, feeling immense pride in their progress. But for families like ours, those moments are bittersweet—because with SPG50, a rare and devastating neurodegenerative disorder, those abilities will slowly be taken away.

SPG50 is relentless. It strips children of their ability to walk, to communicate, and eventually, to care for themselves. It brings seizures, cognitive decline, and a future filled with uncertainty. Our nephew, Jack, was fortunate to receive gene therapy at just five months old, and we've seen the difference it has made in his development. Meanwhile, our niece, Naomi, is still waiting for treatment, and every day, we see the gap between them grow. As an aunt and uncle, it is heartbreaking to know that time is working against her, that what she has worked so hard to learn could be taken away.

Time is not on our side. Every day without action brings us closer to irreversible damage. That is why we are pleading with you—please recognize the urgent need for funding gene therapy programs that offer real hope. Your support could mean the difference between despair and a future where children like Naomi have the chance to keep what they have fought so hard to achieve.

Please, do not let these children be forgotten. Stand with us in this fight.

Best,
Ryan and Emmiley Simmen